

Board of Governors Meeting via Teleconference/Webinar

September 12, 2017

12:00 - 2:00 pm ET



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Introductions

Grayson Norquist, MD, MSPH

Chairperson, Board of Governors

Joe Selby, MD, MPH

Executive Director





Dr. Robert “Bob” Louis Jesse, M.D., Ph.D

It is with great sadness that PCORI learned of the recent death of Dr. Robert Jesse, a distinguished physician, long-time member of the health leadership team at the Department of Veteran Affairs, and an original member of PCORI’s Board of Governors. Bob died of cancer, at home, surrounded by family.

Bob was a great friend of patients, clinicians, and all of us at PCORI who were fortunate to spend time with him. He was an intelligent and warm leader, a caring and tireless mentor, and dedicated in all of his work to improving the lives of patients and their families.

We will all miss Bob’s wise guidance as a member of our Board of Governors; PCORI will miss him as a close friend and colleague. But we’re all grateful that his substantial legacy includes having been one of those who played an essential role in making PCORI what it is today.



Agenda

Time	Agenda Item
12:00	Call to Order, Roll Call, and Welcome
12:00-12:05	Consider for Approval: Minutes of the August 15, 2017 Board Meeting
12:05–12:25	Consider for Approval: Cycle 3 2016 Pragmatic Clinical Studies (PCS) Awards
12:25-12:45	Consider for Approval: Cycle 3 2016 Targeted PFA Awards – Sickle Cell Disease
12:45-1:05	Consider for Approval: Cycle 3 2016 Targeted PFA Awards – Multiple Sclerosis
1:05-1:25	Consider for Approval: Cycle 3 2016 Targeted PFA Award – Opioid Use for Chronic Pain
1:25-1:35	Consider for Approval: Bylaws and Methodology Committee (MC) Charter
1:35-1:40	Consider for Approval: MC Chair and Vice-Chair Nominations

Board Vote

Call for a Motion to:

- **Approve** the Minutes of the August 15, 2017 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



Pragmatic Clinical Studies

Cycle 3 2016 Award Slate

Christine Goertz, DC, PhD
Chair, Selection Committee

Evelyn P. Whitlock, MD, MPH
Chief Science Officer



Pragmatic Clinical Studies – Cycle 3 2016

Merit Review Criteria

1. Potential for the study to fill critical gaps in evidence
2. Potential for the study findings to be adopted into clinical practice and improve delivery of care
3. Scientific merit (research design, analysis, and outcomes)
4. Investigator(s) and environment
5. Patient-centeredness
6. Patient and stakeholder engagement



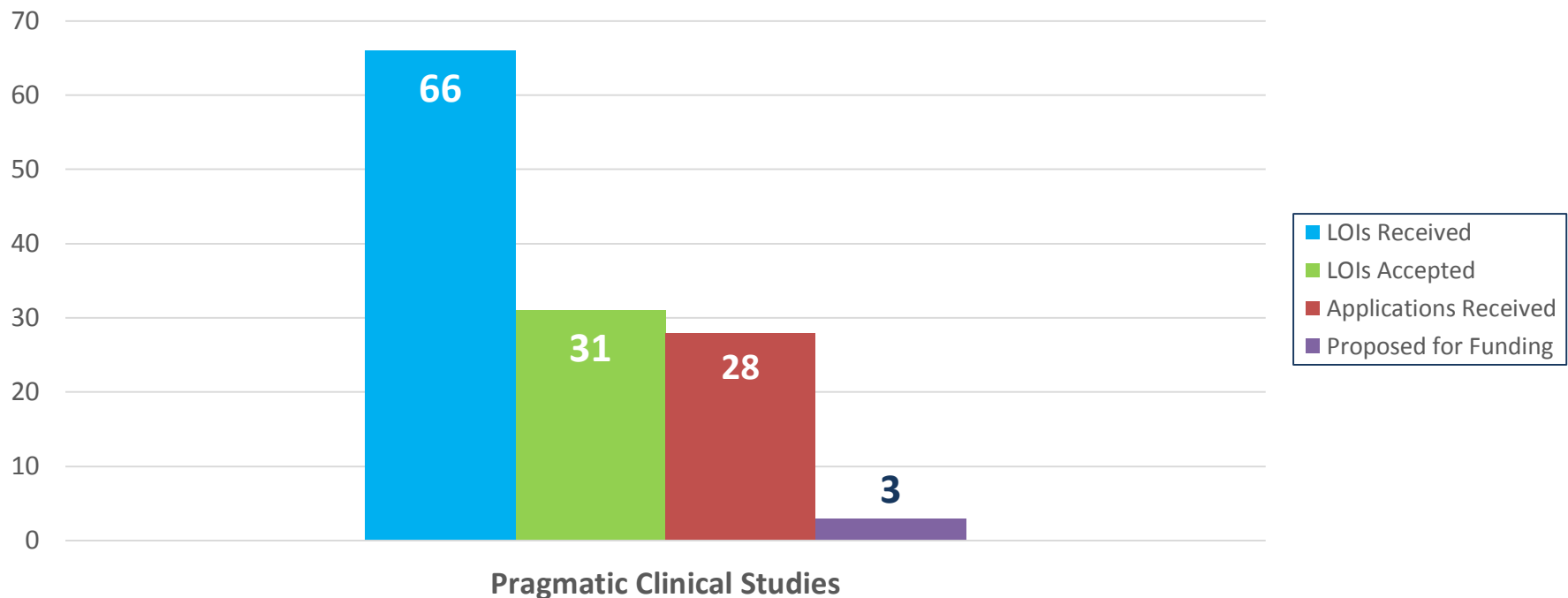
Pragmatic Clinical Studies – Cycle 3 2016

Process Overview

- 66 Letters of Intent (LOIs) submitted
- 31 LOIs invited to submit a full application (**47%**)
- 28 applications were received (**90% of invited LOIs**)

Funding rate is 11 percent

- We are proposing to fund 3 applications* out of 28 received applications



Pragmatic Clinical Studies – Cycle 3 2016

3 Recommended Projects

Project
PRO-ACTIVE: Comparing the Effectiveness of Prophylactic Swallow Intervention for Patients Receiving Radiotherapy for Head and Neck Cancer
PREPARE: Pragmatic Randomized Trial Evaluating Pre-Operative Antiseptic Skin Solutions in Fractured Extremities
Comparative Effectiveness of School-Based Caries Prevention Programs for Children in Underserved, Low Income, Hispanic Communities



Project 1: PRO-ACTIVE: Comparing the Effectiveness of Prophylactic Swallow Intervention for Patients Receiving Radiotherapy for Head and Neck Cancer

- **Research Question:** What is the comparative effectiveness of proactive vs reactive swallowing interventions among patients with head and neck cancer (HNC) planning to undergo radiotherapy (RT)?
- **Population:** Any patient who begins radiotherapy for HNC with a functional swallow at baseline
- **Intervention/Comparator(s):**
 - Pro-Active (high-intensity): swallowing exercises in addition to therapy focused on eating challenging foods, given during the period of RT
 - Pro-Active (low-intensity): therapy focused on eating challenging foods, started prior to RT
 - Re-Active: therapy following the onset of dysphagia
- **Outcomes of Interest:**
 - *Primary:* Duration of feeding tube use post RT
 - *Secondary:* quality of life, functional status and physical dysphagia
- **Study Design:** Randomized controlled trial (RCT)
 - Sample Size: 952 patients at 7 sites
- **Length of Follow-up:** 12 months
- **Duration of Active Intervention:** Approximately 2 months
- **Total Project Cost:** \$8.5M



Project 1: PRO-ACTIVE: Comparing the Effectiveness of Prophylactic Swallow Intervention for Patients Receiving Radiotherapy for Head and Neck Cancer

- **Potential Impact:** Provides better evidence about how to plan rehabilitation services for preventing dysphagia
- **Patient-Centeredness:** Maintaining the ability to swallow foods and liquids is ranked most important by patients with HNC
- **Engagement:** Stakeholders include patients, physicians, allied health providers. Policymakers and payers will convene regularly via quarterly Stakeholder Research Partner Panels



Project 2: PREPARE: Pragmatic Randomized Trial Evaluating Pre-Operative Antiseptic Skin Solutions in Fractured Extremities

- **Research Question:** What is the comparative effectiveness of two pre-operative antiseptic skin solutions for acute extremity fracture surgery?
- **Population:** Patients aged 16+ receiving surgical treatment of an extremity fracture
- **Intervention:** Iodine povacrylex in alcohol
- **Comparator(s):** Chlorhexidine gluconate in alcohol
- **Outcomes of Interest:**
 - *Primary:* Surgical site infection (SSI)
 - *Secondary:* Quality of life (SF-12)
- **Study Design:** Cluster crossover randomized trial
 - Sample Size: 7820 across 10 sites
- **Length of Follow-up:** 90 days
- **Duration of Active Intervention:** Pre-operative application of antiseptic solution
- **Total Project Cost:** \$10.9 M



Project 2: PREPARE: Pragmatic Randomized Trial Evaluating Pre-Operative Antiseptic Skin Solutions in Fractured Extremities

- **Potential Impact:** In compound fractures, the incidence of Surgical Site Infection is high. No previous trials have included patients undergoing fracture surgery
 - New evidence for patients with compound fractures may have a major influence on clinical practice
 - Including patients with closed fractures can provide insight into whether the choice of skin preparation solution is relevant for other types of orthopedic surgery
- **Patient-Centeredness:** Data collection incorporates minimal burden to participants (occurs during routine post-operative follow up visits)
- **Engagement:** The study will leverage the infrastructure of an existing patient engagement program at the host university. The study will engage patient leaders as co-presenters in webinars and live sessions and as co-authors on abstracts and publications



Project 3: Comparative Effectiveness of School-Based Caries Prevention Programs for Children in Underserved, Low Income, Hispanic Communities

- **Research Question:** What is the comparative effectiveness of simple treatment compared to complex treatment in two school-based, caries prevention programs?
- **Population:** Low income Hispanic/Latino elementary school children in a medically underserved area
- **Intervention:** Simple treatment (silver diamine fluoride plus fluoride varnish)
- **Comparator(s):** Complex treatment (traditional sealants and fluoride varnish)
- **Outcomes of Interest:**
 - *Primary:* Prevention of progression of existing caries and new caries prevention
 - *Secondary:* Quality of life, toothaches, school absences, academic performance
- **Study Design:** Cluster randomized controlled trial
 - Sample Size: 14,100 (60 schools)
- **Length of Follow-up:** 2 years for prevention of progression of existing caries; 3 years for new caries prevention
- **Duration of Active Intervention:** One school-based dental visit
- **Total Project Cost:** \$13.4M



Project 3: Comparative Effectiveness of School-Based Caries Prevention Programs for Children in Underserved, Low Income, Hispanic Communities

- **Potential Impact:** The study could lead to higher healthcare quality for patients and sustainable access to dental care for a large underserved minority patient population. If both methods are equally effective, simple prevention could reach four times as many children for the same time and cost as the complex approach
- **Patient-Centeredness:** The study team has involved Latino/Hispanic low-income parents of school age children in the program design since 2013. The proposal incorporates multiple patient-centered requests including: free school-based care, potential color change (silver diamine fluoride on posterior teeth only), improved health, better academic performance, and quality of life
- **Engagement:** Includes patient and stakeholder partners, Hispanic/Latino parents, elementary school faculty, community health workers, city/state agency representatives, and local and national dental insurer representatives. Stakeholders from state and national dental insurers will support state-wide and national dissemination efforts



Pragmatic Clinical Studies – Cycle 3 2016

3 Recommended Projects

PFA	Proposed Total Award*
Pragmatic Clinical Studies	\$32.8M

* 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



Board Vote

Call for a Motion to:

- **Approve** funding for the recommended slate of awards from the Cycle 3 2016 Pragmatic Clinical Studies PFA

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



Management of Care Transitions for Emerging Adults with Sickle Cell Disease

Cycle 3 2016 Award Slate

Christine Goertz, DC, PhD
Chair, Selection Committee

Evelyn P. Whitlock, MD, MPH
Chief Science Officer



Emerging Adults with Sickle Cell Disease – Cycle 3 2016

Objective of the PFA

- **Research Question:** What is the comparative effectiveness of established transition coordination models for emerging adults with Sickle Cell Disease (SCD) transitioning from pediatric to adult care?
- **Population:** Emerging adults (16-25 years of age) with SCD
- Interested in randomized controlled trials comparing interventions with proven efficacy in emerging adults with SCD or other relevant populations
- Demonstrate robust engagement of patients, caregivers, clinicians, and other key stakeholders
- Incorporate patient-centered strategies within a health system setting



Emerging Adults with Sickle Cell Disease – Cycle 3 2016

Merit Review Criteria

1. Potential for the study to fill critical gaps in evidence
2. Potential for the study findings to be adopted into clinical practice and improve delivery of care
3. Scientific merit (research design, analysis, and outcomes)
4. Investigator(s) and environment
5. Patient-centeredness
6. Patient and stakeholder engagement



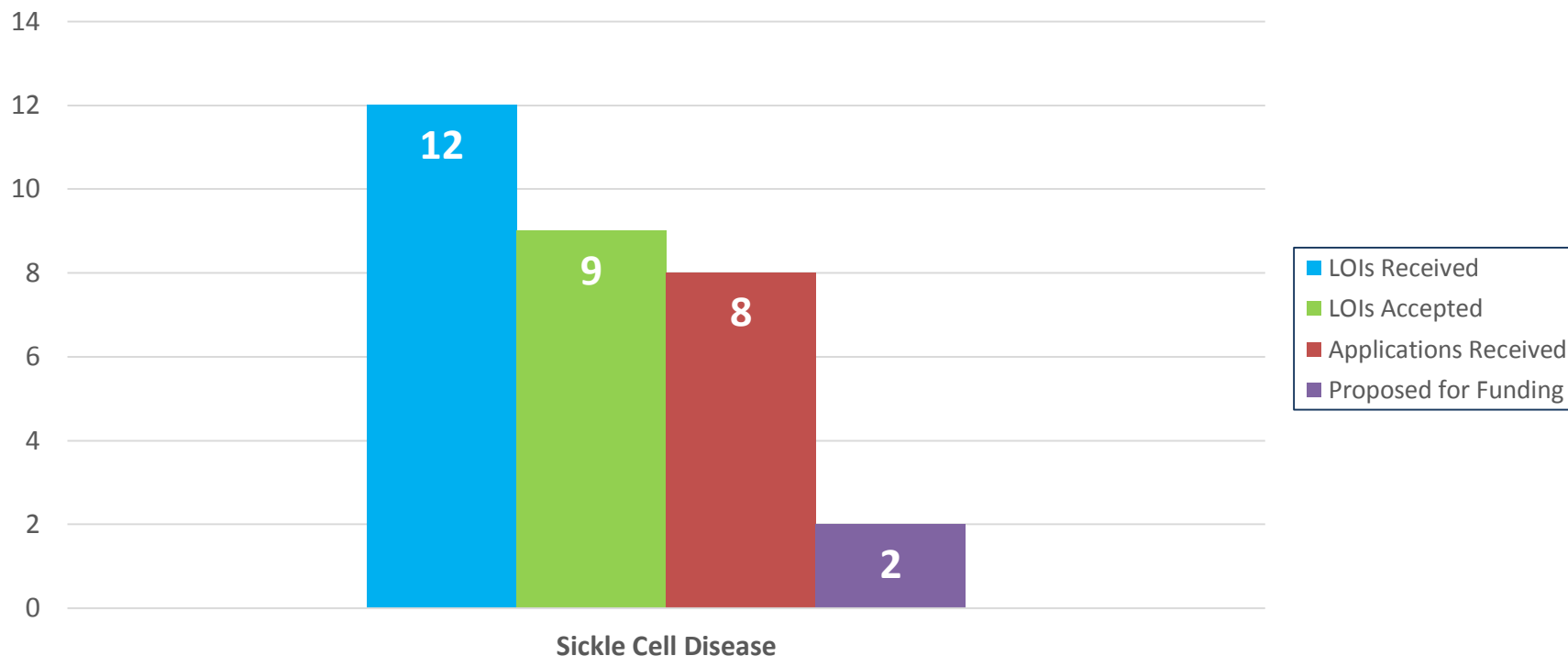
Emerging Adults with Sickle Cell Disease – Cycle 3 2016

Process Overview

- 12 Letters of Intent (LOIs) submitted
- 9 LOIs invited to submit a full application (**75%**)
- 8 applications were received (**89% of invited LOIs**)

Funding rate is 25 percent

- We are proposing to fund 2 applications* out of 8 received applications



Emerging Adults with Sickle Cell Disease – Cycle 3 2016

2 Recommended Projects

Project
Comparative Effectiveness of Peer Mentoring versus Structured Education Based Transition Programming for the Management of Care Transitions in Emerging Adults with Sickle Cell Disease
Community Health Workers and Mobile Health for Emerging Adults Transitioning Sickle Cell Disease Care (COMETS Trial)



Project 1: Comparative Effectiveness of Peer Mentoring versus Structured Education Based Transition Programming for the Management of Care Transitions in Emerging Adults with Sickle Cell Disease

- **Research Question:** “What is the effectiveness of peer mentoring in reducing acute care encounters and acute care reliance rates?”
- **Population:** Emerging adults, 16-25 years of age, with SCD from 14 clinical sites across the Southeastern US
- **Comparators:**
 - Structured Education-based Transition (STE) Programming
 - Peer mentoring in addition to STE
- **Key Outcomes:**
 - *Primary: Acute care utilization and ambulatory care visits*
 - *Secondary: Self-care behavior, quality of life, self-efficacy and satisfaction*
- **Study Design:** Cluster Randomized Controlled Trial
 - Sample Size: 700 (14 clusters)
- **Length of Follow-up:** 24 months
- **Duration of Active Intervention:** 24 months
- **Total Project Cost:** \$9.8M



Project 1: Comparative Effectiveness of Peer Mentoring versus Structured Education Based Transition Programming for the Management of Care Transitions in Emerging Adults with Sickle Cell Disease

- **Potential Impact:**
 - Reduce the high mortality, morbidity, and poor quality of life during transition
 - Identify effective models of healthcare transition in reducing acute care visits and improving patient reported outcomes
- **Patient-Centeredness:**
 - Patients and their caregivers identified the importance of this topic and closing the evidence gap
 - Primary and secondary outcomes were determined to be important to the patient population
- **Engagement:**
 - Partners will be engaged through monthly community advisory board meetings, monthly study advisory council meetings, and annual conferences
 - Local patient engagement activities are also planned every 6-12 months
 - Dissemination to occur through strategies such as social media platforms and a study website, and will involve community churches and events



Project 2: Community Health Workers and Mobile Health for Emerging Adults Transitioning Sickle Cell Disease Care (COMETS Trial)

- **Research Question:** “Can community health workers and mobile applications help me to improve my ability to take care of myself, stay connected with my doctors, avoid visits to the hospital, and ultimately maximize my quality of life while I am transitioning from my pediatric to adult doctors?”
- **Population:** Individuals 17 years of age or older with SCD; will transition within 12 months
- **Comparators:**
 - Enhanced usual care
 - Enhanced usual care plus mHealth self-management
 - Enhanced usual care plus peer community health worker
- **Key Outcomes:**
 - *Primary: Health-related quality of life*
 - *Secondary: Acute health care utilization*
- **Study Design:** 3-arm Randomized Controlled Trial
 - Sample Size: 450
- **Length of Follow-up:** 18 months
- **Duration of Active Intervention:** 6 months
- **Total Project Cost:** \$8.5M



Project 2: Community Health Workers and Mobile Health for Emerging Adults Transitioning Sickle Cell Disease Care (COMETS Trial)

- **Potential Impact:**
 - Make information about transition to adult care readily available to patients, which was identified as a top concern
 - Improve self-efficacy and disease management, which could reduce acute care utilization and rates of mortality
- **Patient-Centeredness:**
 - Trial outcomes based on a survey of emerging adults with SCD
 - Importance of successful transition and increased quality of life were identified by patients and caregivers
- **Engagement:**
 - Multi-stakeholder groups, including patient and family stakeholders, actively planned the study, providing important information and perspectives on relevant trial outcomes, interventions, recruitment and trial procedures
 - Study results will be distributed widely, to young adult patients with SCD and their caregivers, clinical providers, and community organizations, to facilitate their implementation in everyday settings



Emerging Adults with Sickle Cell Disease – Cycle 3 2016

2 Recommended Projects

PFA	Proposed Total Award*
Management of Care Transitions for Emerging Adults with Sickle Cell Disease	\$18.2M

* 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



Board Vote

Call for a Motion to:

- **Approve** funding for the recommended slate of awards from the Cycle 3 2016 Management of Care Transitions for Emerging Adults with Sickle Cell Disease PFA

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



Treatment of Multiple Sclerosis

Cycle 3 2016 Award Slate

Christine Goertz, DC, PhD
Chair, Selection Committee

Evelyn P. Whitlock, MD, MPH
Chief Science Officer



Treatment of Multiple Sclerosis – Cycle 3 2016

Objective of the PFA

PCORI seeks to fund randomized clinical trials or large observational studies that compare two or more alternative clinical strategies for treatment of MS that address the following questions:

1. What are the comparative benefits and harms of different disease-modifying therapies (DMTs) or therapeutic strategies in patients with relapsing, remitting multiple sclerosis on symptoms, functioning, quality of life, disease activity, and disease progression?
2. What are the comparative benefits and harms of different approaches, other than DMTs, for ameliorating important symptoms in people with MS?
3. What is the comparative effectiveness of telerehabilitation versus conventional direct care interventions for improving outcomes in people with MS, such as functional status, fatigue, and quality of life?



Treatment of Multiple Sclerosis – Cycle 3 2016

Merit Review Criteria

1. Potential for the study to fill critical gaps in evidence
2. Potential for the study findings to be adopted into clinical practice and improve delivery of care
3. Scientific merit (research design, analysis, and outcomes)
4. Investigator(s) and environment
5. Patient-centeredness
6. Patient and stakeholder engagement



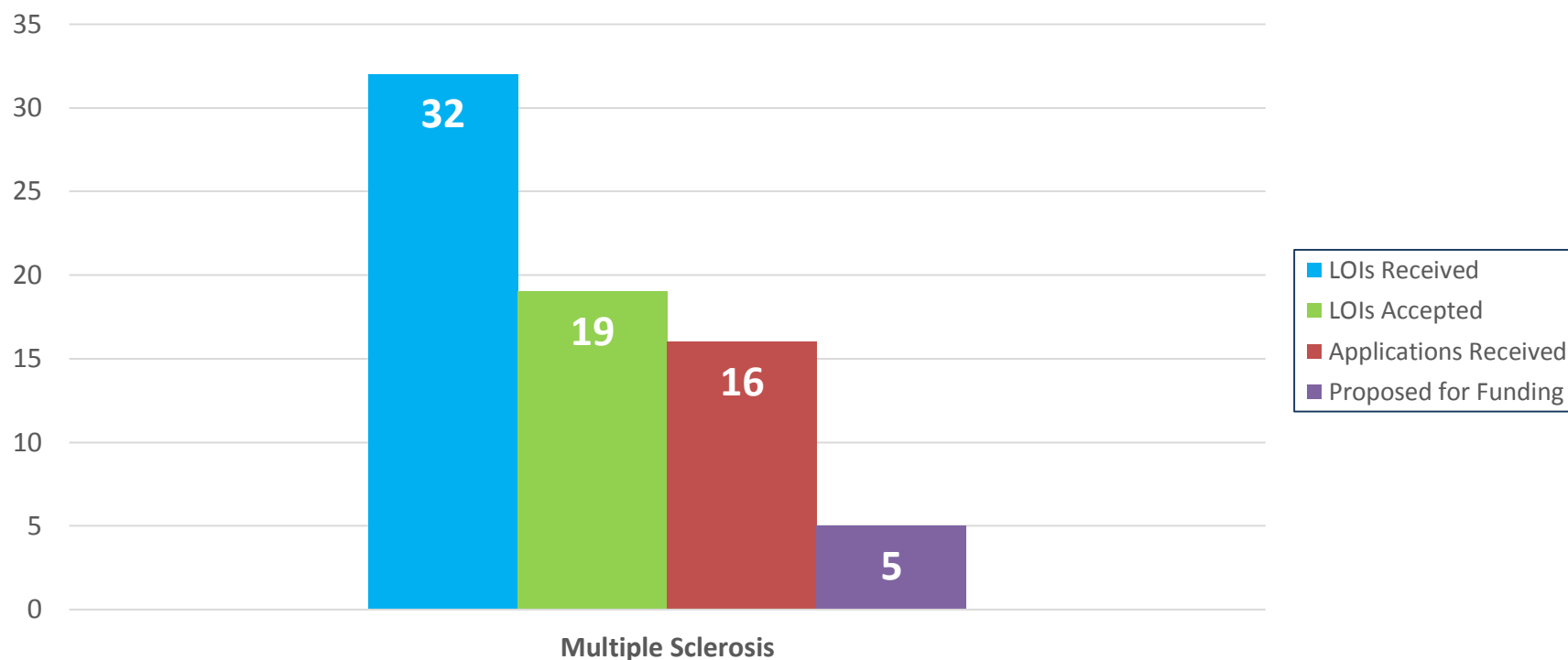
Treatment of Multiple Sclerosis – Cycle 3 2016

Process Overview

- 32 Letters of Intent (LOIs) submitted
- 19 LOIs invited to submit a full application (59%)
- 16 applications were received (84% of invited LOIs)

Funding rate is 31 percent

- We are proposing to fund 5 applications* out of 16 received applications



Treatment of Multiple Sclerosis – Cycle 3 2016

5 Recommended Projects

Project
Determining the Effectiveness of Early Intensive versus Escalation Approaches for the Treatment of Relapsing-Remitting Multiple Sclerosis (Deliver-MS)
A Randomized Controlled Trial of Telephone-Delivered Cognitive Behavioral-Therapy, Modafinil, and Combination Therapy of Both Interventions for Fatigue in Multiple Sclerosis
A Pragmatic Trial to Evaluate the Intermediate-Term Effects of Early, Aggressive versus Escalation Therapy in People with Multiple Sclerosis
Comparative Effectiveness of an Exercise Intervention Delivered via Telerehabilitation and Conventional Mode of Delivery
Comparing the Effectiveness of Fatigue Management Programs for People with MS



Project 1: Determining the Effectiveness of Early Intensive versus Escalation Approaches for the Treatment of Relapsing-Remitting Multiple Sclerosis (DELIVER-MS)

- **Research Question:** What is the comparative effectiveness of an early second-line vs. an escalation DMT approach for persons with relapsing-remitting MS?
- **Population:** Treatment-naïve adults ages 18-55 with relapsing-remitting MS
- **Intervention:** Early second-line treatment (natalizumab, alemtuzumab, or ocrelizumab)
- **Comparator:** Escalation (initially any approved DMT other than these second-line treatments, followed by escalation of therapy as needed to any approved DMT)
- **Outcomes of Interest:**
 - *Primary:* Brain volume loss at 3 years measured by MRI
 - *Secondary:* Disability, function, quality of life, other PROs, medication tolerability
- **Study Design:** Randomized clinical study with concomitant prospective observational study. Sample Sizes: 400 in RCT and 400 in observational study in 16 sites in the US and the UK
- **Length of Follow-up:** 3 years
- **Duration of Active Intervention:** 3 years
- **Total Project Cost:** \$10.6M



Project 1: Determining the Effectiveness of Early Intensive versus Escalation Approaches for the Treatment of Relapsing-Remitting Multiple Sclerosis (DELIVER-MS)

- **Potential Impact:** The two intervention strategies are both in use but widely debated, and there are no data directly comparing their effectiveness. Thus, this study will provide evidence to enable patients and clinicians to make an informed decision about which approach is most appropriate and help patients, clinicians, and payers make informed decisions about medication
- **Patient-Centeredness:** Patients are concerned about brain volume loss, disability, and the other planned outcome measures, and the harmful side effects of medication
- **Engagement:** Patients, clinicians, caregivers, and payers will serve on the study advisory committee and be involved in the planning and conduct of the study. The advisory committee will advise on dissemination, including use of social media, print media, and web media and manuscripts



Project 2: A Randomized Controlled Trial of Telephone-Delivered Cognitive Behavioral-Therapy, Modafinil, and Combination Therapy of Both Interventions for Fatigue in Multiple Sclerosis

- **Research Question:** What is the comparative effectiveness of telephone-delivered CBT vs. modafinil or a combination of both patients with MS experiencing fatigue symptoms?
- **Population:** Adults with MS-related fatigue
- **Interventions/Comparators:** Telephone-delivered CBT, modafinil, combination of both
- **Outcomes of Interest:**
 - *Primary:* Fatigue symptoms (Modified Fatigue Impact Scale)
 - *Secondary:* Fatigue impact severity and Fatigability
- **Study Design:** RCT with sample size of 300 subjects recruited from 2 sites
- **Length of Follow-up:** 12 weeks
- **Duration of Active Intervention:** 12 weeks
- **Total Project Cost:** \$3.5M



Project 2: A Randomized Controlled Trial of Telephone-Delivered Cognitive Behavioral-Therapy, Modafinil, and Combination Therapy of Both Interventions for Fatigue in Multiple Sclerosis

- **Potential Impact:** Will provide information to patients and clinicians about two commonly used approaches for managing fatigue in MS
- **Patient-Centeredness:** Fatigue is the most common symptom reported by patients with MS, affecting up to 90% of patients
- **Engagement:** Stakeholders will include patients, providers, and payers who will participate in study monitoring, dissemination of results, publications and manuscripts. Treatment manuals and protocols will be made available to MS healthcare providers and organizations, targeting patients facing geographic and access barriers to in-person care



Project 3: A Pragmatic Trial to Evaluate the Intermediate-Term Effects of Early, Aggressive versus Escalation Therapy in People with Multiple Sclerosis

- **Research Question:** What is the comparative effectiveness of an early second-line vs. an escalation DMT approach for persons with relapsing-remitting MS?
- **Population:** Treatment-naïve adults ages 18-70 with relapsing remitting MS
- **Intervention:** Early second-line treatment
- **Comparator:** Escalation (initially any approved DMT other than selected second-line treatments, followed by escalation of therapy as needed to any approved DMT)
- **Outcomes of Interest:**
 - *Primary:* Disability progression (increase in expanded disability status scale at 3 and 6 months)
 - *Secondary:* Cognition, fatigue, quality of life
- **Study Design:** RCT, with population stratified by baseline disability risk
 - Sample Size: 900 in 40 sites
- **Length of Follow-up:** 3.5 years
- **Duration of Active Intervention:** 3.5 years
- **Total Project Cost:** \$13.5M



Project 3: A Pragmatic Trial to Evaluate the Intermediate-Term Effects of Early, Aggressive versus Escalation Therapy in People with Multiple Sclerosis

- **Potential Impact:** The two intervention strategies are both in use but widely debated, and there are no data directly comparing their effectiveness. Thus, this study will provide evidence to enable patients and clinicians to make an informed decision about which approach is most appropriate
- **Patient-Centeredness:** Outcomes of disability and quality of life are central concerns of patients with MS
- **Engagement:** Study Advisory Committee will include clinicians, biostatisticians, patients, and payers. Patient partners and stakeholders will assist in generating study recruitment materials and will help identify specific avenues of patient engagement. The research team will help disseminate research findings at local, national, and international conferences



Project 4: Comparative Effectiveness of an Exercise Intervention Delivered via Telerehabilitation and Conventional Mode of Delivery

- **Research Question:** In people with MS, does an individualized exercise program delivered via telerehab yield comparable benefits for walking and mobility and other MS outcomes when compared to clinic-based delivery?
- **Population:** Adults 18 years or older with relapse remitting or progressive MS and moderate disability
- **Intervention:** An evidence-based individualized exercise program delivered via telerehab
- **Comparator:** Clinic-based exercise program
- **Outcomes of Interest:**
 - *Primary:* walking and mobility (timed 25 foot walk test)
 - *Secondary:* disability, pain, fatigue, quality of life
- **Study Design:** Non-inferiority, 2-staged randomized controlled trial
 - Stage 1: patients randomized to 'preference' or 'randomization' condition
 - Stage 2: patients assigned to tele or clinic delivery by patient preference or randomization
 - Sample size: 500
- **Length of Follow-up:** 12 months
- **Duration of Active Intervention:** 16 weeks
- **Total Project Cost:** \$5.7M



Project 4: Comparative Effectiveness of an Exercise Intervention Delivered via Telerehabilitation and Conventional Mode of Delivery

- **Potential Impact:** Results may increase access to effective interventions to improve mobility and decrease disability for patients with MS
- **Patient-Centeredness:** The research question, interventions, and outcomes were informed by patients and caregivers
- **Engagement:** Patients with MS and their caregivers, community medical, rehabilitation and exercise professionals have advised on the study proposal; will continue to participate as Advisory Board members, along with payers. The Advisory Board will guide targeted dissemination to stakeholder groups and stimulate sustainable community exercise programs; focus groups at each study site will be involved in development dissemination plans and training materials. Several national organizations will assist with dissemination to rehabilitation and exercise professionals, and researchers



Project 5: Comparing the Effectiveness of Fatigue Management Programs for People with MS

- **Research Question:** Do teleconference, internet, and in-person delivery of a fatigue self-management course provide similar outcomes for fatigue and its impact on physical, mental, and social function?
- **Population:** Adults 18 years or older with relapse remitting or progressive MS, with fatigue
- **Intervention:** Occupational-therapist-led fatigue self-management course, “Managing Fatigue” (MF)
- **Comparator(s):** Delivery of MF course by (1) group teleconference, (2) group online delivery using website, (3) one-on-one in-person
- **Outcomes of Interest:**
 - *Primary:* fatigue
 - *Secondary:* pain, health-related quality of life, social function
- **Study Design:** RCT
 - Sample Size: 610
- **Length of Follow-up:** 6 months
- **Duration of Active Intervention:** Varies based on intervention arm
- **Total Project Cost:** \$4.9M



Project 5: Comparing the Effectiveness of Fatigue Management Programs for People with MS

- **Potential Impact:** Results may help address geographical, individual, and condition-specific barriers, and increase access to effective treatment for an important MS symptom (fatigue)
- **Patient-Centeredness:** Study topic, outcomes, protocol are patient-centered
- **Engagement:** Focus groups with patients with MS and caregivers were conducted to inform study design and outcomes. Patients and stakeholders will be included in the Study Advisory Committee. Implementation and dissemination will occur through Town Hall meetings with researchers, research participants, patients with MS, and community leaders; resources will be shared via public domain websites to facilitate adoption and ensure reproducibility



Treatment of Multiple Sclerosis – Cycle 3 2016

5 Recommended Projects

PFA	Proposed Total Award*
Treatment of Multiple Sclerosis	\$38.1M

* 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



Board Vote

Call for a Motion to:

- **Approve** funding for the recommended slate of awards from the Cycle 3 2016 Treatment of Multiple Sclerosis PFA

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



Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain

Cycle 3 2016 Award Slate

Christine Goertz, DC, PhD
Chair, Selection Committee

Evelyn P. Whitlock, MD, MPH
Chief Science Officer



Long-Term Opioid Use for Chronic Pain – Cycle 3 2016

Objective of the PFA

- PCORI re-opened the announcement on Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain
- Goal: To fund studies that compare two or more alternatives for addressing the management and reduction of long-term opioid use for chronic pain
- **Priority Research Questions:**
 1. Among patients with chronic non-cancer pain on moderate/high-dose long-term opioid therapy, what is the comparative effectiveness of strategies for reducing/eliminating opioid use while managing pain?
 2. Among patients with chronic non-cancer pain on moderate/low-dose long-term opioid therapy, what are the comparative effectiveness and harms of strategies used to limit dose escalation?



Long-Term Opioid Use for Chronic Pain – Cycle 3 2016

Merit Review Criteria

1. Potential for the study to fill critical gaps in evidence
2. Potential for the study findings to be adopted into clinical practice and improve delivery of care
3. Scientific merit (research design, analysis, and outcomes)
4. Investigator(s) and environment
5. Patient-centeredness
6. Patient and stakeholder engagement



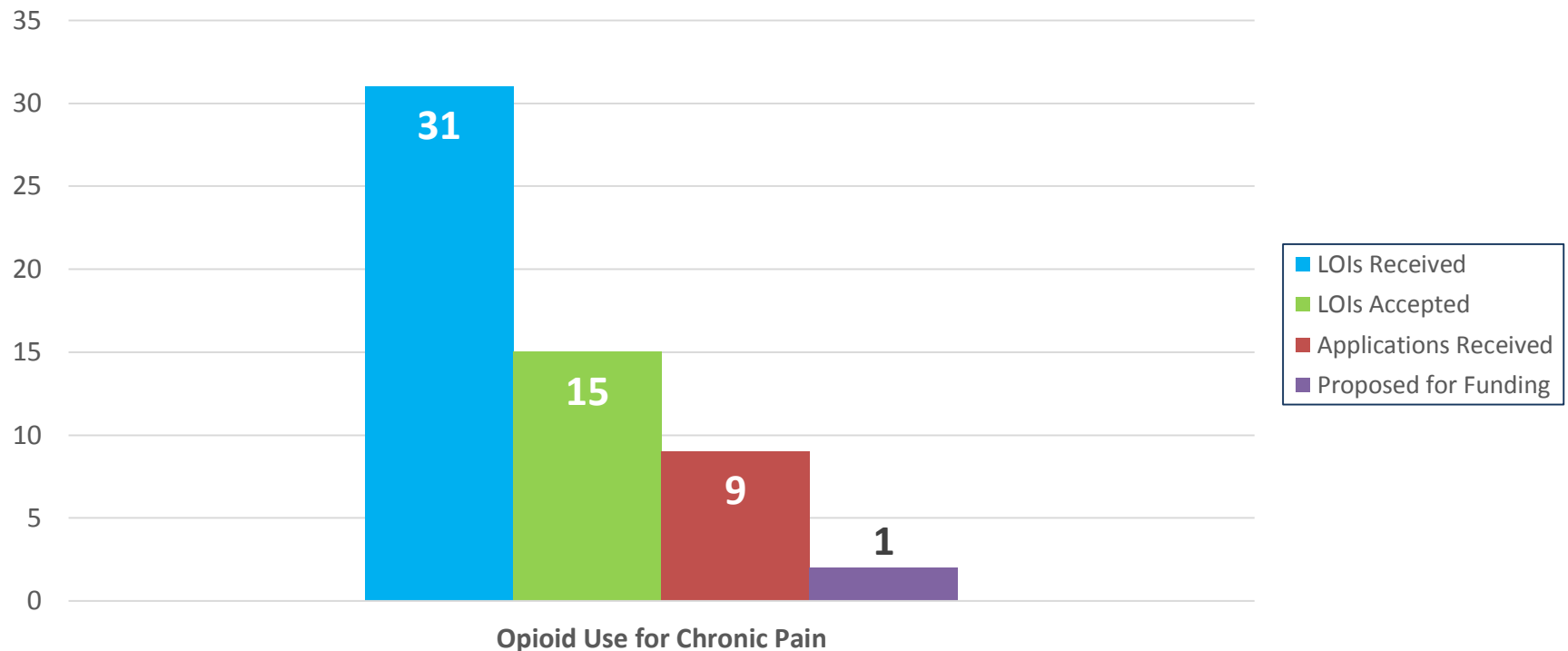
Long-Term Opioid Use for Chronic Pain – Cycle 3 2016

Process Overview

- 31 Letters of Intent (LOIs) submitted
- 15 LOIs invited to submit a full application (**48%**)
- 9 applications were received (**60% of invited LOIs**)

Funding rate is 11 percent

- We are proposing to fund 1 application* out of 9 received applications



Long-Term Opioid Use for Chronic Pain – Cycle 3 2016

1 Recommended Project

Project
Comparative Effectiveness of Cognitive Behavioral Therapy and Chronic Pain Self-Management within the Context of Opioid Reduction

Comparative Effectiveness of Cognitive Behavioral Therapy and Chronic Pain Self-Management within the Context of Opioid Reduction



Project 1: Comparative Effectiveness of Cognitive Behavioral Therapy and Chronic Pain Self-Management within the Context of Opioid Reduction

- **Research Question:** What is the comparative effectiveness of Cognitive Behavioral Therapy (CBT) versus Chronic Pain Self-Management Program (CPSMP) care, within the context of a patient-centered opioid taper pathway?
- **Population:** Adults (aged 18 to 85) with chronic non-cancer pain receiving prescription opioids (>10 morphine equivalent daily dose) for more than 3 months
- **Intervention:** CBT within the context of a patient-centered opioid taper
- **Comparator(s):**
 1. Chronic Pain Self-Management Program within the context of a patient-centered taper
 2. Patient-centered taper alone
- **Outcomes of Interest:**
 - *Primary:* 50% reduction in opioid dosage and no increased pain at 12 months
 - *Secondary:* pain interference, physical function, depression, fatigue, pain perception, role function
- **Study Design:** RCT
 - Sample Size: 865 in 5 sites
- **Length of Follow-up:** 12 months
- **Duration of Active Intervention:** 8 weeks for CBT and 6 weeks for Chronic Pain Self-Management Program
- **Total Project Cost:** \$8.8M



Project 1: Comparative Effectiveness of Cognitive Behavioral Therapy and Chronic Pain Self-Management within the Context of Opioid Reduction

- **Potential Impact:** Study has the capacity to provide important information to patients and physicians as to which behavioral treatment strategy improves patient outcomes while opioids are reduced
- **Patient-Centeredness:** Study engages participants through a patient-centered collaborative taper
- **Engagement:** The Study Advisory Committee includes 8 members with experience as a patient or family member, CPSMP Peer Leaders, and CBT psychologists. Study results will be disseminated through patient and health professional webinars, regional conferences, press releases, video clips, and a patient-centered opioid reduction toolkit



Long-Term Opioid Use for Chronic Pain – Cycle 3 2016

1 Recommended Project

PFA	Proposed Total Award*
Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain	\$8.8M

* 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



Board Vote

Call for a Motion to:

- **Approve** funding for the recommended slate of awards from the Cycle 3 2016 Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain PFA

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



Proposed Amendments to PCORI Bylaws and Methodology Committee Charter

Kerry Barnett, JD

Chair, Governance Committee

Nadine Peters, JD, MPH

Deputy General Counsel



Proposed Amendments to PCORI Bylaws and Methodology Committee (MC) Charter

- Bylaws and MC Charter currently limit the terms of the Chair and Vice Chair of Methodology Committee to two 2-year terms
- Terms of the current MC Chair and Vice Chair will expire on September 26, 2017
 - This is the expiration of their second term, and according to current Bylaws and MC Charter, they have reached their term limits
- Long standing principles based on PCORI Conflict of Interest Policies have limited members serving as Methodology Committee Chair and Vice Chair to members who have declared themselves ineligible to apply for PCORI funding
- Currently only three members of the Methodology Committee, in addition to the NIH and AHRQ Director designee members, have declared themselves ineligible for PCORI funding
 - Governance Committee noted that generally NIH and AHRQ Director designee members would not be included in the pool of possible Chairs and Vice Chairs given their primary focus on their agencies



Governance Committee Recommendation

Governance Committee has the responsibility of making nominations for the position of Chair and Vice Chair of the Methodology Committee to the Board. After much discussion, the Governance Committee recommends:

- that the Board amend the PCORI Bylaws and the Methodology Committee Charter to retain current term limits for the MC Chair and Vice Chair; and,
- that the Board add language amending the Bylaws and Methodology Committee Charter allowing the Board to approve an extension of the terms of the MC Chair and Vice Chair upon recommendation of the Governance Committee



Other Proposed Amendments to PCORI Bylaws

The Governance Committee reviewed other provisions of PCORI Bylaws, and recommends the following additional amendments to the PCORI Bylaws to be consistent with other governing documents:

- Section 5.5(b)(iii): Amendment to align the Bylaws with recently approved changes to the Governance Committee Charter relating to communication with GAO;
- Section 5.8(a)(ii): Amendment to align the Bylaws with approved changes to the EDIC Charter relating to areas of EDIC's oversight;
- Section 5.8 (c), 5.8(d): Amendments to align the Bylaws with Board's adoption of the Executive Committee charter;
- Section 10.2: Amendment to change the section title to capture the full language in this provision, which addresses both capital expenditures *and investments*



Board Vote

Call for a Motion to:

Approve proposed amendments to the PCORI Bylaws and Methodology 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**

Roll Call Vote:

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



Nomination for Methodology Committee Chair and Vice Chair

Kerry Barnett, JD

Chair, Governance Committee



Motion for MC Chair, Vice Chair Nominations

If the Board approves the proposed revisions to the Methodology Committee Charter and the PCORI Bylaws, Governance Committee recommends approval of the following:

- Nomination by the Governance Committee for Robin Newhouse, PhD, RN, to serve as Chair, and Steve Goodman, MD, MHS, PhD, to serve as Vice Chair, of the Methodology Committee for an additional two-year term following the expiration of their second two-year term of service as Chair and Vice Chair



Board Vote

Call for a Motion to:

Approve the recommendation of an additional two-year term for Robin Newhouse to serve as Chair, and Steve Goodman to serve as Vice Chair of the Methodology Committee.

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



Wrap Up and Adjournment

Grayson Norquist, MD, MSPH
Chairperson, Board of Governors

