PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Board of Governors Meeting via Teleconference / Webinar

Tuesday, September 12, 2017

12:00 pm – 2:00 pm ET

Board Members Present:

Grayson Norquist, MD, MSPH, *Chairperson*; Kerry Barnett, JD, *Vice Chairperson;* Debra Barksdale, PhD, RN; Larry Becker; Michael Lauer, MD (Acting as Designee for Francis Collins, MD, PhD); Alicia Fernandez, MD; Christine Goertz, DC, PhD; Leah Hole-Marshall, JD; Russell Howerton, MD; Gopal Khanna; Richard Kuntz, MD, MSc; Sharon Levine, MD; Freda Lewis-Hall, MD; Ellen Sigal, PhD; Kathleen Troeger, MPH; Robert Zwolak, MD, PhD

Board Members Absent:

Allen Douma, MD; Gail Hunt; Harlan Krumholz, MD, SM; Barbara McNeil, MD, PhD

MINUTES

Agenda Item	**PCORI Speakers**
Call to Order, Roll Call, and Welcome - *Grayson Norquist, MD, MSPH, Chairperson* and *Joe Selby, MD, MPH, Executive Director*	Dr. Norquist opened the meeting, thanked all who joined the call and welcomed all to the meeting of the PCORI Board of Governors. He then read the Chair Statement on Conflict of Interest. Dr. Norquist reflected on the recent death of Dr. Robert Jesse, a PCORI Board member who had served on PCORI's Board since its inception. After a moment of silence to reflect on Dr. Jesse's life and passing, Board members offered their thoughts on Dr. Jesse's contributions and kindness, describing him as courageous and inspirational. The PCORI Board members and Dr. Selby joined in offering their condolences to all of Dr. Jesse's family, colleagues and friends. After this solemn tribute to Dr. Jesse, Dr. Selby reviewed the agenda for the meeting.
Consider for Approval: Minutes of the August 15, 2017 Board Meeting - *Grayson Norquist, MD, MSPH*	*The following motion was made by Kerry Barnett and seconded by Kathleen Troeger:* **Motion:** Approve the minutes of the August 15, 2017 Board of Governors meeting. *Approved by a majority vote of the Board by voice vote (no opposed; no abstentions)*
Consider for Approval: Proposed Slate for Cycle 3, 2016 Pragmatic Clinical Studies (PCS) Awards - *Christine Goertz, DC, PhD, Chair, Selection Committee and Evelyn Whitlock, MD, MPH, Chief Science Officer*	Dr. Goertz, after thanking all applicants and awardees, introduced Dr. Whitlock who began her presentation by introducing the proposed slate of awards for the Cycle 3, 2016 Pragmatic Clinical Studies PFA. The goal of the PFA is to fund pragmatic clinical trials, large simple trials, or large-scale observational studies that compare two or more alternatives for addressing prevention, diagnosis, treatment, or management of a disease or symptom; improving healthcare system-

	level approaches to managing care; or for eliminating health or healthcare disparities. Of the 66 Letters of Intent submitted, 31 (47%) were invited to submit a full application. Of these, 28 applications were submitted. The Selection Committee recommended that the Board approve funding a slate of three applications for a proposed total commitment of $32.8M of the posted $90M. The slate consisted of the following three research projects: • 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 The first research project is a pragmatic randomized controlled trial with the aim to study the comparative effectiveness of proactive versus reactive swallowing interventions among patients with head and neck cancer who are planning to undergo radiotherapy. The total project cost is $8.5M. The second research project is a pragmatic cluster crossover randomized trial to study the comparative effectiveness of two pre-operative antiseptic skin solutions for acute extremity fracture surgery. The total project is $10.9M. The third research project is a cluster randomized controlled trial with the aim to study the comparative effectiveness of simple treatment compared to complex treatment in two school-based, caries prevention programs within low-income Hispanic/Latino elementary children in a medically underserved area. The total project cost is $13.4M. The Board asked whether the first project relating to head and neck cancer studies would study the efficacy of radiotherapy, in addition to quality of life issues for patients. Dr. Whitlock noted the project is not looking at the efficacy of radiotherapy, per se, but that, within the context of radiotherapy, the study aims to understand how to address outcomes that will keep patients functional and able to maintain their swallowing. *The following motion was made by Russell Howerton and seconded by Ellen Sigal:* **Motion:** Approve funding for the recommended slate of awards from the Cycle 3, 2016 Pragmatic Clinical Studies PFA. *Approved by a majority vote of the voting Board members by roll call*

	(14 in favor; 0 opposed; 0 abstentions and 1 recusal, Robert Zwolak); Christine Goertz was not present during this vote.
Consider for Approval: Proposed Slate for Cycle 3, 2016 Targeted PFA Award • Management of Care Transitions for Emerging Adults with Sickle Cell Disease - *Christine Goertz, DC, PhD and Evelyn Whitlock, MD, MPH*	Dr. Whitlock continued her presentation by introducing the proposed slate of awards for the Cycle 3, 2016 Management of Care Transitions for Emerging Adults with Sickle Cell Disease PFA. The goal of the PFA is to fund strong proposals that focus on the following research question: What is the comparative effectiveness of established transition coordination models for emerging adults with Sickle Cell Disease transitioning from pediatric to adult care? Of the 12 Letters of Intent submitted, 9 (75%) were invited to submit a full application. Of these, 8 applications were submitted. On July 13, 2017, the Selection Committee recommended to fund two applications for a proposed total commitment of $18.2M out of the posted $25M. The slate consisted of the following two research projects: • 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) The first research project is a cluster randomized controlled trial that aims to study the effectiveness of peer mentoring in reducing acute care encounters and acute care reliance rates within emerging adults, 16-25 years of age, with Sickle Cell Disease from 14 clinical sites across the Southeastern United States. The total project cost is $9.8M. The second research project is a 3-arm, randomized controlled trial to compare the effectiveness of two self-management support interventions (community health workers programs and mobile health applications) versus enhanced usual care to improve health-related quality of life and acute care use for transitioning youth with Sickle Cell Disease. The total project cost is $8.5M. *The following motion was made by Michael Lauer and seconded by Larry Becker:* **Motion:** 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. *Approved by a majority vote of the voting Board members by roll call (14 in favor; 0 opposed; 0 abstentions and 1 recusal, Russell Howerton); Robert Zwolak was not present during this vote.*
Consider for Approval: Proposed Slate for Cycle 3, 2016 Targeted PFA Award	Dr. Whitlock introduced the proposed awards for the Cycle 3, 2016 Treatment of Multiple Sclerosis PFA. This PFA is a repost from Cycle 3, 2015, approved by the Board September 28, 2015.

• Treatment for Multiple Sclerosis - *Christine Goertz, DC, PhD and Evelyn Whitlock, MD, MPH*	The goal of the PFA is to fund randomized controlled trials or observational studies that compare two or more alternatives for the treatment of Multiple Sclerosis, with a focus on the effects of therapies on the symptoms experienced by MS patients and on quality of life and functional status. Of the 32 Letters of Intent submitted, 19 (59%) were invited to submit a full application. Of these, 16 applications were submitted. On July 13, 2017, the Selection Committee recommended to fund five applications for a proposed total commitment of $38.1M out of the posted $30M. The slate consisted of the following five research projects: • 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 Tele rehabilitation and Conventional Mode of Delivery • Comparing the Effectiveness of Fatigue Management Programs for People with MS The first research project is a multicenter randomized clinical trial that aims to study the comparative effectiveness of an early second-line versus an escalation disease modifying treatment approach (DMT) for treatment-naïve adults ages 18-55 with relapsing remitting MS. The total project cost is $10.6M. The second research project is a randomized controlled trial that aims to compare the effectiveness of a commonly used behavioral treatment strategy (cognitive behavioral therapy), a commonly used medication (modafinil), and a combination of both therapies for fatigue in a large, representative group of patients with Multiple Sclerosis. The total project cost is $3.5M. The third research project is a pragmatic, randomized controlled trial that aims to study the comparative effectiveness of an early second-live versus an escalation DMT approach for treatment-naïve adults ages 18-70 with relapsing-remitting Multiple Sclerosis. The total project cost is $13.5M. The fourth research project is a noninferiority, randomized controlled trial that aims to study whether an individualized exercise program delivered via tele rehabilitation yields comparable benefits for walking, mobility, and other MS outcomes, when compared to clinic-based

	delivery, for adults 18 years or older with relapsing-remitting or progressive MS and moderate disability. The total project cost is $5.7M. The fifth research project is a randomized controlled trial to study whether teleconference, internet, and in-person delivery of a fatigue self-management course provides similar outcomes for fatigue, and its impact on physical, mental, and social function, for adults 18 years or older with relapse remitting or progressive MS, with fatigue. The total project cost is $4.9M. Dr. Whitlock noted the proposed total commitment for the Treatment of Multiple Sclerosis PFA exceeds the original posted amount by $8M. The Selection Committee was comfortable with the overage given that the overall budget for the Cycle 3, 2016 funding cycle was not exceeded. The Board posed questions about the slate, including regarding whether the second project included a placebo. Staff responded the second research project does not include placebo control and the study will be comparing active interventions – cognitive behavioral therapy alone, and modafinil, which is a commonly used medication, and a combination of these two. The investigators are looking at active comparisons, and the combination of those, in terms of the outcomes. *The following motion was made by Larry Becker and seconded by Debra Barksdale:* **Motion:** Approve funding for the recommended slate of awards from the Cycle 3, 2016 Treatment of Multiple Sclerosis PFA. *Approved by a majority vote of the voting Board members by roll call (12 in favor; 0 opposed; 0 abstentions and 3 recusals, Michael Lauer, Alicia Fernandez and Richard Kuntz); Kathleen Troeger was not present during this vote.*
Consider for Approval: Proposed Slate for Cycle 3, 2016 Targeted PFA Award • Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain - *Christine Goertz, DC, PhD and Evelyn Whitlock, MD, MPH*	Dr. Whitlock began her final presentation by introducing the slate of proposed awards for the Cycle 3, 2016 Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain PFA. This PFA is a repost from Cycle 3, 2015 approved by the Board on September 28, 2015. The goal of the PFA is to fund studies that compare two or more alternatives for addressing the management and reduction of long-term opioid use for chronic pain. Of the 31 Letters of Intent submitted, 15 (48%) were invited to submit a full application. Of these, 9 applications were submitted. On July 14, 2017, the Selection Committee recommended to fund one application for a proposed total commitment of $8.8M out of the posted $19M. The slate consisted of one research project:

	• Comparative Effectiveness of Cognitive Behavioral Therapy and Chronic Pain Self-Management within the Context of Opioid Reduction The research project is a multi-site, randomized controlled trial that aims to study the comparative effectiveness of cognitive behavioral therapy versus chronic pain self-management program care, within the context of a patient-centered opioid taper pathway for adults (aged 18 to 85) with chronic non-cancer pain receiving prescription opioids for more than three months. The total project cost is $8.8M. The Board asked whether the project would capture the disease setting as chronic pain and pain management is dependent on the disease setting. . Dr. Whitlock noted since the project is a pragmatic study, there will be no effort to limit to certain disease arenas except non-cancerous. The study will capture disease states and comorbidities. The Board also noted that various subgroups may have different outcomes. Dr. Whitlock commented that the study will consider different subgroups in the heterogeneity of treatment effect analysis. *The following motion was made by Sharon Levine and seconded by Russell Howerton:* **Motion:** 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. *Approved by a majority vote of the voting Board members by roll call (13 in favor; 0 opposed; 0 abstentions and 1 recusal, Freda Lewis-Hall); Gopal Khanna and Kathleen Troeger were not present during this vote.*
Consider for Approval: Proposed Amendments to Bylaws and Methodology Committee Charter - *Kerry Barnett, JD, Chair, Governance Committee and Nadine Peters, JD, MPH*	Mr. Barnett, after presenting the proposed amendments to the Bylaws and Methodology Committee recommended by the Governance Committee, introduced Nadine Peters, Deputy General Counsel, to summarize the background and proposed amendments. Ms. Peters noted that currently the Bylaws and Methodology Committee Charter limit the terms of the Chair and Vice Chair of the Methodology Committee to two consecutive two-year terms, and that Dr. Newhouse and Dr. Goodman will reach their term limits as Chair and Vice Chair (respectively) at the end of September 2017. This prompted discussion by the Governance Committee and Methodology Committee about how to meet PCORI's needs and commitment to strong Methodology Committee leadership while retaining PCORI's longstanding conflict of interest principles that limit eligibility to serve as Chair and Vice Chair to those members who have declared themselves ineligible to apply for PCORI funding. Typically, the Methodology Chair and Vice Chair are required to participate in confidential discussions about research agendas, funding announcements and other matters that could create a conflict of interest should they be eligible to apply for PCORI funds. The Governance Committee, recognizing that term limits can provide

	an appropriate governance opportunity to consider PCORI's needs for the Methodology Committee, recommended that the term limits for the Chair and Vice Chair be retained. However, given that at any given time, the pool o Methodology Committee members who are eligible to serve as Chair and Vice Chair may vary, the Governance Committee recommended that the Bylaws and Methodology Committee Charter be revised to enable the Board, upon recommendation of the Governance Committee, to extend the terms of a current Chair and/or Vice Chair beyond the term limits. She also summarized other proposed amendments to the Bylaws, which are to conform the Bylaws to other governing documents and current practice. *The following motion was made by Kerry Barnett and seconded by Freda Lewis-Hall:* **Motion:** Approve the proposed amendments to the PCORI Bylaws and Methodology Committee Charter. *Approved by a majority vote of the voting Board members by roll call (13 in favor; 0 opposed; 0 abstentions and 0 recusals); Christine Goertz, Gopal Khanna and Kathleen Troeger were not present during this vote.*
Consider for Approval: Methodology Committee Chair and Vice Chair Nominations - *Kerry Barnett, JD*	Mr. Barnett noted that, in light of the approval by the Board of the recommended amendments to the Bylaws and the Methodology Committee Charter, the Governance Committee nominated Robin Newhouse to serve an additional term as Chair of the Methodology Committee and Steve Goodman to serve an additional term as Vice Chair of the Methodology Committee. *The following motion was made by Kerry Barnett and seconded by Debra Barksdale:* **Motion:** 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 *Approved by a majority vote of the Board by voice vote (no opposed; no abstentions)* *Dr. Selby, on behalf of PCORI, expressed gratitude to Dr. Newhouse and Dr. Goodman for agreeing to serve PCORI for an additional two years in these positions.*
Wrap up and Adjournment - *Grayson Norquist, MD, MSP*	Meeting ended at 1:29 pm

Minutes were approved by the PCORI Board of Governors 09-26-2017