Patient-Centered Outcomes Research Institute Board of Governors Sessions

The Westin Crystal City
1800 Jefferson Highway
Arlington, VA 22202

October 30-31, 2017

Board Members Present:

Grayson Norquist, MD, MSPH, *Chairperson*; Debra Barksdale, PhD, RN; Kerry Barnett, JD, *Vice Chairperson;* Larry Becker; Allen Douma, MD (via phone); Alicia Fernandez, MD; Christine Goertz, DC, PhD; Leah Hole-Marshall, JD; Russell Howerton, MD, FACS; Gopal Khanna, MBA; Harlan Krumholz, MD, SM (via phone) Michael Lauer, MD (representing Francis S. Collins, MD, PhD); Sharon Levine, MD; Freda Lewis-Hall, MD; Barbara McNeil, MD, PhD; (via phone); Robert Zwolak, MD, PhD

Board Members Absent:
Gail Hunt; Richard Kuntz, MD, MSc; Ellen Sigal, PhD; Kathleen Troeger, MPH

Staff:
Joe Selby, MD, MPH, *Executive Director*; Evelyn P. Whitlock, MD, MPH; Jean Slutsky, PA, MSPH; Regina Yan, MA; Mary Hennessey, Esq.; Michele Orza, ScD

Agenda Items	Related Minutes
Welcome and Call to Order **Consider for Approval:** • Minutes of September 26, 2017 Board Meeting - *Grayson Norquist, MD, MSPH, Chairperson*	Dr. Norquist chaired and opened the meeting, welcomed all to the meeting of the PCORI Board of Governors and read the Chair Statement on Conflict of Interest. *The following motion was presented to the Board for approval.* **Motion:** Approve the minutes of the September 26, 2017 Board of Governors meeting. *Approved by a majority vote of the voting Board members as presented. Debra Barksdale, Harlan Krumholz, and Barbara McNeil were not present for this vote.*
Executive Director's Report and Q3 Dashboard Review - *Joe Selby, MD, MPH, Executive Director*	Dr. Selby presented on the evolution of the PCORI funded portfolio, and updates to the PCORI website that allow updated features for searching of publications and funded projects. Visitors to the website can now search for results of PCORI-funded studies. He provided an overview of the upcoming 2017 Annual Meeting, which will have eight breakout sessions featuring research results. Dr. Selby presented the Q3-2017 Dashboard for the Board's review. The Dashboard demonstrated 5 green-flagged items (items that are on track) and 1 yellow-flagged item (items that are off target by more than 10%-15%, depending on the metric). The metric for PCORI Peer Review is yellow because early data show that the process is taking longer to complete than the established target. There were no red-flagged items (items that are off-target). Dr. Selby presented examples of results on PCORI's strategic goals: To increase information, to speed uptake, and to influence research. He presented results of a PCORI-funded research study of the effect of glucose monitoring in patients with non-insulin treated diabetes. This results publication had a very high Altmetric score (a measure of attention), and had been cited in at least 120 news stories, demonstrating broad interest. To provide an example of uptake of study results, Dr. Selby shared that 3 publications about PCORI-funded studies have been taken up into UpToDate®, and that there are two new PCORI CME/CE activities based on results of PCORI-funded studies. He shared that PCORI is credited with inspiring the creation of a Patient and Family Advisory Council for the Cancer Outcomes Research Program at Massachusetts General Hospital. Dr. Selby described that there are 7 publications about PCORI funded projects from Q3 with very high Altmetric scores

	(>100), demonstrating that PCORI-funded research is capturing public attention. Dr. Selby provided an overview of the status of recruitment of ongoing PCORI-funded research projects. He shared that 164 research projects have completed recruitment. Of these studies, 69% of research projects completed recruitment on time or early, and that 96% achieved greater than 90% of their target enrollment. Dr. Selby discussed the roles of contract modifications and extensions. He explained that the average time to complete recruitment was 130% of the planned recruitment timeline. 38% of funded studies had an extension of their timeline, and 10% of funded studies had a modification to their enrollment target. Board Members expressed interest in the ability to compare our portfolio to the NIH, both project monitoring and requirements for making results available to the public. It was suggested that there are opportunities for speeding up PCORI Peer Review, to make results of PCORI-funded research available sooner.
Methodology Committee Update **Consider for Approval**: • Release New Methodology Standards for Public Comment - *Robin Newhouse, PhD, RN, Chair*	Dr. Robin Newhouse, Chair of the PCORI Methodology Committee (MC), began her presentation by giving an overview on the Methodology Committee and PCORI's Methodology Standards. PCORI's Methodology Standards are required by PCORI's authorizing law and represent minimal standards for design, conduct, and reporting of comparative effectiveness research and patient-centered outcomes research. The MC proposed five new standards to be posted for public comment. The Standards for Studies of Complex Interventions (4 standards) represent a new category of standards. The Data Management Plan standard will be added to the existing set of Standards for Data Integrity and Rigorous Analyses. If the Board approves the release of the proposed Methodology Standards, they would be made available on PCORI's website for public comment. Comments would then be read, processed and categorized, and if indicated, the MC would revise the proposed Methodology Standards based on public comments and recommend them to the Board for final adoption. The expected timeline to complete these next steps is until May 2018. Board members discussed whether the MC considered enhancing the standards by incorporating more specific details into the standards to provide additional guidance.

	Dr. Steve Goodman, Vice Chair of the MC, provided data preservation as an example of the difficulty in being too prescriptive, in that there are many ways that data preservation can be appropriately handled. Dr. Goodman also indicated that feedback regarding particular standards that are difficult to interpret and may not be as helpful would be considered valuable. *The following motion was made by Christine Goertz and seconded by Larry Becker:* **Motion:** Approve release for public comment of the proposed new Methodology Standards *Approved by a majority vote of the voting Board members (13 in favor; 0 opposed; 0 abstentions and 0 recusals)*; *Debra Barksdale, Harlan Krumholz, and Barbara McNeil were not present for this vote.*
• Methodology Committee Efforts to Improve the Science and Methods of PCOR/CER	Dr. Newhouse gave an overview of the Methodology Committee (MC) and highlighted the MC's role in meeting its legislative mandate to develop and improve the science and methods of comparative clinical effectiveness research. The MC was established under PCORI's authorizing law to provide guidance and recommendations to the Board of Governors and PCORI regarding methods for patient-centered outcomes research. These recommendations and guidance include the appropriate use of methods in PCOR, methodological standards, and identifying and addressing gaps in research methods or their application. The MC's contributions to PCORI and the broader research community include formative work in advising on PCOR and research priorities and the development of the Methodology Report & Methodology Standards. Future initiatives include examining PCORI's portfolio to identify opportunities for improving the quality of evidence generated by clinical research. In addition to these activities, Dr. Newhouse also discussed the work of the Clinical Trials Advisory Panel (CTAP), over which the MC has oversight. During the discussion, Leah Hole-Marshall suggested that the MC consider methodology standards for N-of-1 trials. Dr. Goodman also indicated that the process, between developing standards and improving the quality of science, encompasses many steps and that the MC's path moving forward is to look at those steps and recommend which ones PCORI might play a role.

"Physician and Policymaker Perspective on Discussing the Value and Importance of PCORI" **The Honorable J. Phillip Gingrey, MD** Senior Advisor, District Policy Group US Representative (GA-11, 2003 – 2015)	Dr. Gingrey, who served in Congress during the debates on the legislation that included the authorization of PCORI, delivered remarks to the Board outlining his concerns at that time regarding the formation of a federally-funded CER institute. Those concerns revolved largely around CER being used by the government to intrude on the doctor-patient relationship. He went on to express his current satisfaction that PCORI steered clear of those concerns. In particular, he expressed support for the Institute's focus on important clinical topics and the engagement of patients and clinicians. In a discussion with the Board, he provided his perspective on key communities that may serve as effective communicators of the importance and strength of PCORI's work toward improving healthcare for patients.
Consider for Approval: Additional Application from the Cycle 3, 2016 Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain PFA - *Christine Goertz, DC, PhD, Chair, Selection Committee and Evelyn Whitlock, MD, MPH, Chief Science Officer*	Dr. Whitlock began her presentation by introducing the proposed additional award for the Cycle 3, 2016 Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain PFA. On September 12, 2017, one application, from the Opioid Use for Chronic Pain PFA, was proposed to the Board and approved for funding for a total commitment of $8.8M out of the budgeted $19M. In addition to these applications, the Selection Committee (SC) also identified one application for further programmatic review. After further programmatic review, the SC approved staff's recommendation that the Board fund the following additional application: • Integrated Health Services to Reduce Opioid Use while Managing Chronic Pain This research project is a randomized, controlled trial that aims to study the comparative effectiveness of two approaches to managing chronic non-cancer pain patients who are on chronic opioid therapy: a guideline-concordant pharmacotherapy-approach integrated with shared decision-making (SDM) (Arm 1) compared with a guideline-concordant pharmacotherapy approach integrated with motivational interviewing (MI) and cognitive behavioral therapy for chronic pain (Arm 2). The proposed total commitment for the Cycle 3, 2016 Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain PFA, which includes the research project already approved by the Board and this proposed application, is $17.8M out of the budgeted $19M.

	The following motion was made by Michael Lauer and seconded by Leah Hole-Marshall: **Motion:** Approve funding for the recommended additional award 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 voice vote (12 in favor; 0 opposed; Freda Lewis-Hall abstained; and 0 recusals); Debra Barksdale, Harlan Krumholz, and Barbara McNeil were not present for this vote.*
PCORI at 7 Years: Strategy Committee Reports on Accomplishments to Date and Looking to the Future **Individual Committee Reports and Q&A** **Goal 1 – Increasing Information:** Science Oversight Committee (SOC) - *Robert Zwolak, MD, PhD, Chair; Alicia Fernandez, MD, Vice Chair; Evelyn Whitlock, MD, MPH*	Dr. Whitlock discussed the state of PCORI's research enterprise in 2017, which is under the oversight of the Science Oversight Committee. Focused-funding opportunities are a growing investment for critical clinical topics and PCORI continues to develop its funded portfolio by seeking out new research areas from stakeholders and working internally to identify evidence gaps within topics of interest to patients. The multi-pronged approach to improving PCORI's research productivity includes: increasing the number of Pragmatic Clinical Studies (PCS) PFAs from two to three per year, adding special emphasis topics to PCS PFAs, continuing to develop new targeted PFAs, and monitoring research allocations within all programs and to revise as needed. In FY2016, $310M of research awards were announced and in FY2017, planned research awards are $382M. Between 2017-2019, current areas of continued focused topic development include: anxiety in youth, atrial fibrillation, type 2 diabetes treatments, and targeted PFA re-postings. Adjustments have also been made to the Peer Review process to maintain high quality reports, while reducing the number of revisions to the Draft Final Research Report (DFRR) that a PI must complete, as well as the overall length of time in Peer Review. Thus far in 2017, 100 DFRRs have been submitted. Strategies for the future include: working with others in PCORI to enhance the communication and dissemination of the research portfolio, continuing to engage stakeholders in building a robust CER pipeline for future high-priority investments, cementing approaches for heterogeneity of treatment effects for the field and PCORI's future, ensuring that new

	scientific investments over the new few years are as high priority as possible, and ensuring that PCORI's scientific investments realize their contribution to applicable useful knowledge through the successful oversight of our research investments. **********
Goal 2 – Speeding Uptake: Engagement, Dissemination, and Implementation Committee - *Debra Barksdale, PhD, RN, Chair; Jean Slutsky, PA, MSPH, Chief Engagement and Dissemination Officer*	The Board received a report on the oversight activities of the Engagement, Dissemination, and Implementation Committee (EDIC), with Ms. Slutsky providing remarks on behalf of Dr. Barksdale. The EDIC's mission and goals align with PCORI Strategic Goal 2: *Speed the implementation and use of patient-centered outcomes research evidence*. The EDIC's four key responsibilities are to establish PCORI as a thought leader in CER and PCOR; ensure stakeholders have mechanisms to engage; ensure efficiency and quality of public reporting; and, dissemination and implementation of PCORI-funded findings. Ms. Slutsky highlighted activities overseen by the EDIC that aim to accomplish each of these goals, including those undertaken by staff in the Public and Patient Engagement, Engagement Awards, Training, Communications, and Dissemination and Implementation teams. The presentation included case studies demonstrating how activities undertaken to meet these responsibilities are leading to close, productive relationships with key stakeholder organizations. A particular focus of the Board's questions related to EDIC's approach to dissemination: EDIC will continue its efforts to be responsive to the guidance of the Board, patients, and stakeholders in dissemination and implementation strategy. **********
Goal 3 – Influencing Research: Research Transformation Committee - *Freda Lewis-Hall, MD, Chair; Allen Douma, MD, Vice Chair; Joe Selby, MD, MPH*	Dr. Selby provided an overview of the Research Transformation Committee's (RTC) responsibilities and efforts to-date. The RTC is responsible for advancing PCORI's Strategic Goal 3 *Influencing clinical and health care research funded by others to be more patient-centered*. In support of its mission, a set of initiatives were embraced by the RTC to influence research funded by others to become more patient-centered. These included funding the development of a patient-centered research infrastructure that could be used by researchers and other funders; championing open science so that results are available more rapidly and broadly; attending to training the workforce in doing PCOR; embedding research within health care systems and co-funding research with other organizations. The presentation outlined the vision, work conducted to advance, and future direction of each initiative.

	The RTC will continue its efforts to influence research funded by other to be more patient-centered.
Moderated Board Discussion: - *Grayson Norquist, MD, MSPH and Kerry Barnett, JD, Vice Chair*	Following the reports on PCORI at 7 Years by the Science Oversight Committee (Goal 1: Increasing Information), Engagement Dissemination, and Implementation Committee (Goal 2: Speeding Uptake), and Research Transformation Committee (Goal 3: Influencing Research), Dr. Norquist proposed discussion questions related to each strategy committee. The discussion of Goal 1 was centered around PCORI's approach to determining when PCORI has maximized its impact and investment in a research area. The Board expressed interest in conducting evidence reviews before considering how much more to invest in targeted funding areas where PCORI already has a large portfolio. The discussion of Goal 2 was centered around the strategy for PCORI's dissemination and implementation activities. The Board expressed that the strategy around dissemination of research findings is specific to each study result, but there are opportunities to be consistent across studies. The discussion of Goal 3 was centered around how to prioritize the five ongoing initiatives to transform research (Open Science, Funding Partnerships, Workforce Training, Methodology Standards Dissemination, and PCORnet). The Board expressed interest in close monitoring of PCORnet initiatives. They also discussed ways to leverage the overlaps of the five initiatives.
Public Comment - *Kristin Carman, MA, PhD, Director, Public and Patient Engagement*	No members of the public requested the opportunity to make a public comment. Thus, there were no public comments presented during this time.
Wrap-up and Adjourn Meeting of the Board - *Grayson Norquist, MD, MSPH*	Meeting ended at 4:53 pm

Minutes were approved by the PCORI Board of Governors 11-21-2017