Board of Governors Meeting

via Teleconference/Webinar

March 25, 2014

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

Patient-Centered Outcomes Research Institute

Welcome and Introductions

Grayson Norquist, MD, MSPH

Chair, Board of Governors

Joe Selby, MD, MPH

Executive Director

Agenda

Time	Agenda Item
12:00 – 12:10 p.m.	Call to Order and Welcome • Minutes from 2/25 Meeting • Withdrawal of Outdated Committee Charters
12:10– 12:15 p.m.	Revisions to Research Transformation Committee Charter Approved 2/25/14
12:15 – 12:25 p.m.	Revised Institutional Policies: • Whistleblower • Record Retention
12:25 – 12:55 p.m.	New Advisory Panel Slates: • Clinical Trials Advisory Panel • Rare Disease Advisory Panel
12:55 – 1:00 p.m.	Wrap up and Adjournment

Board Vote: February 25, 2014 Board Meeting Minutes

Call for a Motion to:	• **Approve** February 25, 2014 Board Meeting Minutes
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

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Withdrawal of Outdated Committee Charters

Joe V. Selby, MD, MPH
Executive Director, PCORI

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Withdrawal of Outdated Charters

- The Board's February 25, 2014 approval of charters for several new committees and revised charters for some existing committees rendered several committees outdated.
- The Board is therefore asked to sunset these committees and approve the withdrawal of their applicable charters:
  - Communications, Outreach, and Engagement Committee (COEC)
  - Program Development Committee (PDC)
  - Nominating Committee
  - Standing Committee on Conflict of Interest (SCCOI)

Board Vote: Approve Withdrawal of Charters

Call for Motion to:

- **Approve withdrawal of Charters for these Committees:**
  - **Communications, Outreach, and Engagement Committee**
  - **Program Development Committee**
  - **Nominating Committee**
  - **Standing Committee on Conflict of Interest**

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**

Revisions to Charter: Research Transformation Committee

Freda Lewis-Hall, MD
Chair, Research Transformation Committee

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Revisions to Charter: Research Transformation Committee

- The Research Transformation Committee (RTC) proposes revisions to its charter, which was approved by the Board on February 25, 2014. The proposed revisions are primarily to reflect the overall purpose of the RTC in more detail:

The Research Transformation Committee (the "Committee") of the Patient-Centered Outcomes Research Institute ("PCORI") shall advise and assist the Board of Governors and PCORI on *encouraging clinical and health care research, including research funded by others, to be more patient-centric by promoting open science, the development of transformative research platforms, and the conduct of more patient-centric and methodologically rigorous research*, as set forth in the "Responsibilities" section of this Charter.

Board Vote: Approve the Revised Charter

Call for Motion to:	• **Approve** the Charter of the Research Transformation Committee as revised
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

Approve Revised Whistleblower and Record Retention Policies

Regina Yan, MA
Chief Operating Officer, PCORI

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Revised Whistleblower Policy

- The revisions (FAC comments incorporated) are intended to reflect the following:
  - That an employee can first discuss claims with his/her supervisor;
  - That claims may be made not only to the Director of HR and Administration, but also to the Chief Operating Officer or the General Counsel;
  - That the General Counsel or the General Counsel's designee will investigate claims;
  - Refined language around how a claim will be resolved; and
  - Clarified the definition of "claim" to tie to illegal or unethical activities.

Board Vote: Approve Revised Whistleblower Policy

Call for a Motion to:	• **Approve** Revised Whistleblower Policy
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

Revised Record Retention Policy

- The revisions (FAC comments incorporated) are intended to reflect the following:
  - Updating timeframes to reflect more specific time periods where appropriate;
  - Updating finance timeframes to align with best practices; and
  - Adding specification to contracts and unfunded application protocols.

- Policy will be reviewed periodically for necessary updates.

Board Vote: Approve Record Retention Policy

Call for a Motion to:	• **Approve** Revised Record Retention Policy
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

Presentation of Proposed Advisory Panel Members

Joe V. Selby, MD, MPH
Executive Director, PCORI

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Meeting Topics and Objectives

What are we going to cover today?

Advisory Panels Overview	• Key information regarding the establishment of PCORI's two new Advisory Panel on Clinical Trials and on Rare Disease
Proposed Advisory Panel Members	• Review and approval of the proposed Advisory Panel members

Advisory Panel Establishment Process

1 Staff Drafts and Submits an Advisory Panel Charter

- Board, MC, and/or PCORI staff identify the need to establish an Advisory Panel
- Staff initiates request for an advisory panel by submitting a panel-specific charter

2 Board Reviews the Proposed Advisory Panel Charter

- Board may authorize charter
- Board may request revisions to the charter

3 Staff Activates Application and Selection of Panel Members

- Staff initiates open call for applications, via the PCORI website and other communications
- Applicants submit an application via the PCORI Web site OR 3rd party organization submits nomination
- Staff evaluates applicants, per evaluation criteria unique to the panel charter

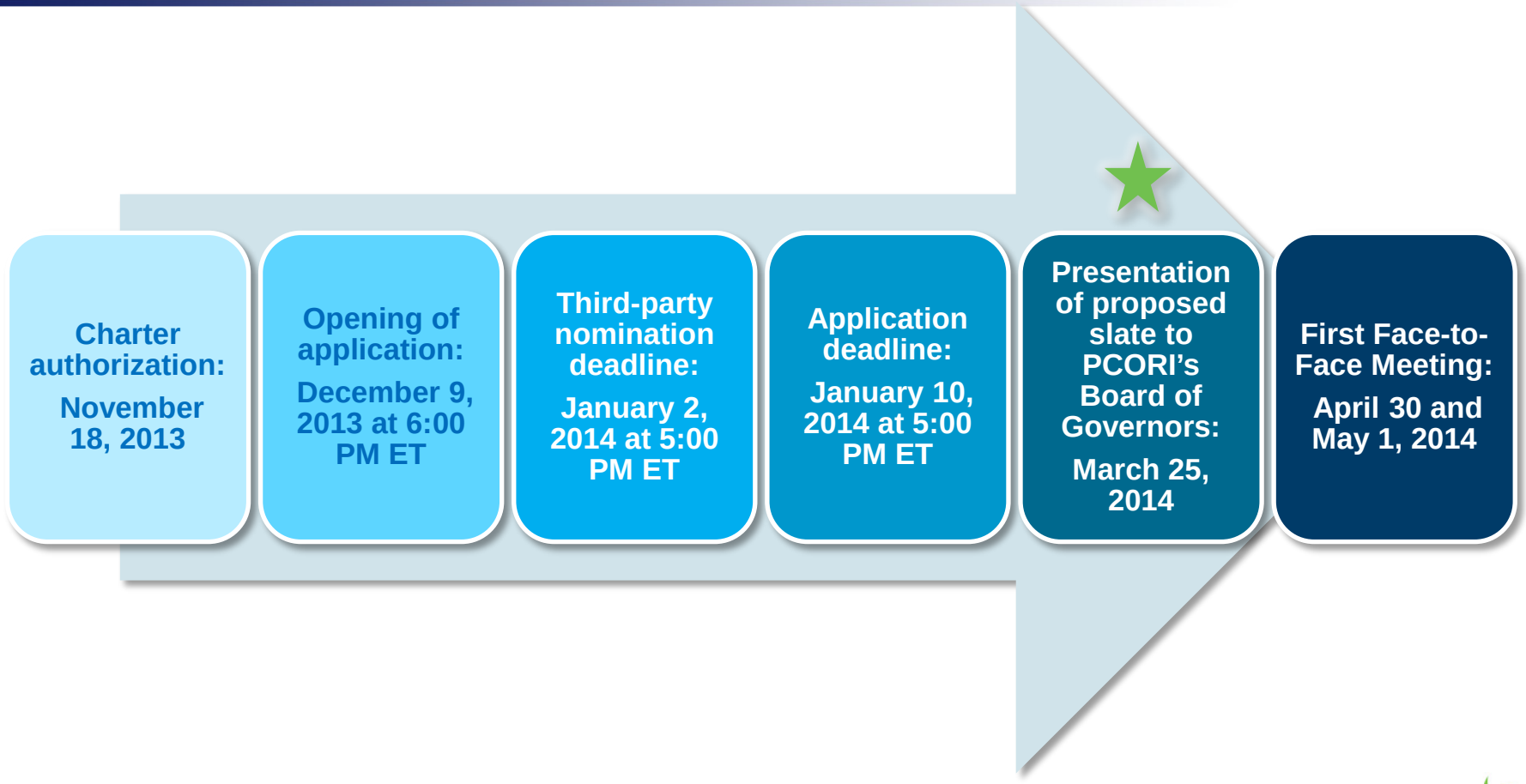
4 Board Approves Advisory Panels

- Staff selects and proposes a panel roster vetted with the Methodology Committee (CTAP) and the Science Oversight Committee (CTAP and RDAP)
- Board authorizes and approves the panel roster

Staff Phase *Board Phase*

Getting Up to Speed on PCORI's Two New Advisory Panels

Timeline



Openings and Applications Received

Advisory Panel	Openings	Applications Received
Clinical Trials	**10-14**	**231**
Rare Disease	**12-15**	**129**

Getting Up to Speed on PCORI's Two New Advisory Panels

What do I need to know?

Legislative Authorization: What does the law say about these new advisory panels?

- PCORI shall appoint expert advisory panels in carrying out randomized clinical trials to advise PCORI on technical questions that may arise during the conduct of such research.
- PCORI shall appoint an expert advisory panel for purposes of assisting in the design of the research study for rare diseases and determining the relative value and feasibility of conducting the research study.

Purpose: What is the purpose of these new advisory panels?

- The Advisory Panel on Clinical Trials will support our methodological work by providing expertise throughout the selection, design, and implementation of trials.
- The Advisory Panel on Rare Disease will provide recommendations in two broad areas: the conduct of patient-centered comparative clinical effectiveness research in rare diseases and coordination and engagement with the rare-disease research community.

Getting Up to Speed on PCORI's Two New Advisory Panels

What do I need to know?

Composition

- **Clinical Trials:** 10 to 14 members: At least two members will be patients, caregivers, or representatives of patient advocacy organizations. One member will have special expertise in the ethical dimensions of clinical trials. At least half the panel will be selected from technical experts in the conduct of clinical trials. Consistent with the legislative mandate, the remainder will include other methodologists and individuals not already represented, including practicing and research clinicians, experts in scientific and health services research, health services delivery, and, as appropriate integrative health and primary prevention strategies. Members may also include technical, pharmaceutical, device, and other manufacturer or medical technology experts.
- **Rare Disease:** 12 to 15 members: One-third will be patients, caregivers, or representatives of rare disease advocacy organizations. Consistent with the legislative mandate, the remainder will include representation by practicing and research clinicians, experts in scientific and health services research, health services delivery, and evidence-based medicine, insurers, the life sciences industry, and, as appropriate experts in integrative health and primary prevention strategies. Members may also include other stakeholders, such as representatives of employers, and policy makers.

Terms

- Terms will be staggered so not all members rotate off the panel simultaneously.
- Initially, half the members will be appointed for a one-year term and half appointed to a two-year term. Thereafter, terms for all members will be for two years. Members may not serve for more than two terms.

Proposed Advisory Panel Members

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Advisory Panel Members for Consideration

- Proposed slates will be presented and voted on, one at a time, in the following order:
  - Advisory Panel on Clinical Trials
  - Advisory Panel on Rare Disease

Clinical Trials

Patients, Caregivers, and Patient Advocates

Name	Primary Affiliation	Brief Bio	Term (in Years)
Sanford Jeames	N/A	Patient advocate, protocol reviewer and community health educator with extensive experience and knowledge working with minority populations, under-represented and under-served.	1
Margo Michaels	Education Network to Advance Cancer Clinical Trials (ENACCT)	The Founder of the Education Network to Advance Cancer Clinical Trials (ENACCT) and a national expert in community-based education efforts around cancer clinical research.	2

Clinical Trials
Researchers, Biostatisticians

Name	Primary Affiliation	Brief Bio	Term (in Years)
Jason Connor	Berry Consultants	Leading expert of Bayesian and adaptive trial design.	2
Elizabeth Stuart	American Statistical Association (nomination)	Academic statistician with expertise in randomized trials, including handling complexities such as missing data, clustering, mediation analysis, and noncompliance.	1

Clinical Trials
Researchers, Epidemiologists

Name	Primary Affiliation	Brief Bio	Term (in Years)
Anne McTiernan	Fred Hutchinson Cancer Research Center	Researcher with expertise in clinical trials on disease prevention through weight control, physical activity, and chemoprevention.	1

Clinical Trials

Researchers, Clinical Trialists

Name	Primary Affiliation	Brief Bio	Term (in Years)
Craig Nichols	VMMC testicular cancer clinic and SWOG	Cancer clinical trial development researcher with expertise in medical informatics, clinical decision support and Novel Patient and provider engagement.	1
Frank Rockhold	GlaxoSmithKline	Leader of global organizations in research and development. Including clinical trial design, data standards, benefit to risk, clinical research, epidemiology, and mostrecently pharmacovigilance.	1
Robert Temple	Food and Drug Administration	As FDA administrator, advised drug companies on the conduct of their trials and reviewing the results of those trials.	2

Clinical Trials

Researchers, Experts in Scientific and Health Services

Name	Primary Affiliation	Brief Bio	Term (in Years)
Merrick Zwarenstein	University of Western Ontario	Health services physician and PI with a particular interest in development and evaluation of large scale health service interventions in the real world using mixed methods and including pragmatic randomized trials.	2

Clinical Trials

Researchers, Experts in Ethical Dimensions of Clinical Trials

Name	Primary Affiliation	Brief Bio	Term (in Years)
John Lantos	Children's Mercy Hospital	Pediatrician, bioethicist, and leader in academic medicine.	2

Clinical Trials
Alternates

Name	Primary Affiliation	Stakeholder Group	Brief Bio	Term (in Years)
Linda Morgan	Mission Hospitals	Patients, Caregivers, and Patient Advocates	Active patient advocate who was diagnosed with Parkinson's disease eight years ago who has participated over 25 trials.	1 or 2
Daniel Merenstein	Georgetown University	Researcher, Clinical Trialist	Clinician researcher with expertise in effectiveness research, or research occurring in real-life situations.	1 or 2

Clinical Trials
Alternates, cont.

Name	Primary Affiliation	Stakeholder Group	Brief Bio	Term (in Years)
Niteesh Chouhdry	Harvard Medical School	Researcher, Expert in Scientific and Health Services Research	Internist and health services researcher whose work focuses on the clinical and economic consequences of using evidence-based therapies for the management of common chronic conditions	1 or 2
Roy Poses	Miskatonic Group	Researcher, Expert in Scientific and Health Services Research	Researcher with interests in evidence-based medicine and medical decision making.	1 or 2

Board Vote: Approve Proposed Slates

Call for a Motion to:	• **Approve** the proposed slate of members of the Advisory Panel on Clinical Trials
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

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Rare Disease
Patients, Caregivers, and Patient Advocates

Name	Primary Affiliation	Brief Bio	Term (in Years)
Jacqueline Alikhaani	American Heart Association	Heart patient and survivor of a rare congenital heart disease misdiagnosed for 48 years.	1
Vincent Del Gaizo	Friends of CARRA	Father of a systematic onset juvenile idiopathic arthritis patient who is actively involved in the field of pediatric rheumatology research for the past 12 years.	2
Kate Lorig	N/A	Born with Gaucher disease and cancer survivor.	2
Marc Skinner	National Hemophilia Foundation	Hemophilia patient and active national and international patient advocate.	1
Russell Teagarden	National Organization for Rare Disorders (NORD)	SVP of Medical and Scientific Affairs at The National Organization for Rare Disorders.	2

Rare Disease
Clinicians

Name	Primary Affiliation	Brief Bio	Term (in Years)
Marilyn Bull	American Academy of Pediatrics (nomination)	Pediatric expert in related child health and rare diseases.	1
Mardi Gomberg-Maitland	Pulmonary Hypertension Association (nomination)	Expert clinician and researcher in the field of pulmonary vascular disease with a focus on pulmonary arterial hypertension (PAH).	2
Marshall Summar	Children's National Medical Center	Geneticist and pediatrician with expertise in rare diseases.	1

Rare Disease
Researchers

Name	Primary Affiliation	Brief Bio	Term (in Years)
Sindy Escobar Alvarez	Doris Duke Charitable Foundation	Clinical research expertise in sickle cell disease.	1
Yaffa Rubenstein	National Institutes of Health	Researcher and director at the Office for Rare Diseases Research including directing the Global Rare Disease Patient Registry Data Repository (GRDR) program.	2

Rare Disease
Industry

Name	Primary Affiliation	Brief Bio	Term (in Years)
Philip Ruff	Shire Pharmaceuticals	Industry background in the rare disease drug pipeline.	2
James Wu	Hyperion Therapeutics	Industry background in academic/clinical medicine and expertise in market access, contracting, and health economics and orphan drug development.	1

Rare Disease
Payers

Name	Primary Affiliation	Brief Bio	Term (in Years)
Uday Deshmukh	**Florida Blue**	**Payer perspective with additional international health experience, public health training and many years of health policy experience.**	**1**

Rare Disease
Alternates

Name	Primary Affiliation	Stakeholder Group	Brief Bio	Term (in Years)
Josh Mailman	NorCal CarciNET Community	Patients, Caregivers, and Patient Advocates	Personal history of rare Neuroendocrine and patient advocate for access to improved diagnostics and treatment options.	1 or 2
Marc Williams	Geisinger Health System	Researchers	Pediatric geneticist and researcher.	1 or 2
Josephine Li-McLeod	Baxter Healthcare	Industry	Extensive experience in industry and academia in health economics and medical outcomes research in rare and orphan disorders.	1 or 2

Board Vote: Approve Proposed Slates

Call for a Motion to:	• **Approve** the proposed slate of members of the Advisory Panel on Rare Disease
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

Wrap-up and Adjournment

Grayson Norquist, MD, MSPH
Chair, Board of Governors

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