



Clinical Trials Advisory Panel Rare Disease Advisory Panel

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PCORI Board of Governors Meeting
Atlanta, GA
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Patient-Centered Outcomes Research Institute

Session Topics and Objectives

What are we going to cover today?

PCORI Advisory Panel Charters

- Review key information regarding the role and establishment of PCORI's Advisory Panels on Clinical Trials and Rare Disease

New Charters

- Presentation of new Charters as endorsed by relevant committees and request **Board vote for approval**

Timeline & Next Steps

- Timeline and next steps for the launch of both Advisory Panels

Clinical Trials Advisory Panel

Background

- Draft Charter discussed at the September Board meeting
 - Recommendation: the Clinical Trials Advisory Panel should work directly with the Methodology Committee
- The charter was revised to reflect that it works directly with the Methodology Committee
- Changes have been approved by the Methodology Committee and Program Development Committee

Board Vote:

Clinical Trials Advisory Panel Charter

Call for a Motion to:

- **Approve** the charter for the Clinical Trials Advisory Panel

Call for the Motion to Be Seconded:

- **Second** the Motion
 - If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion

Vote:

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

Rare Disease Advisory Panel

Applicable Legislative Provisions

(4)(A)(iii) EXPERT ADVISORY PANEL FOR RARE DISEASE.

In the case of a research study for rare disease, the Institute shall appoint an expert advisory panel for purposes of **assisting in the design of the research study** and **determining the relative value and feasibility** of conducting the research study.

Composition of the Panel:

- Required representatives:
 - Patients, caregivers, or representatives of rare disease advocacy organizations
 - Practicing and research clinicians
 - Scientific and health services research, health services delivery, and evidence-based medicine
- Additional possible membership:
 - Experts in integrative health and primary prevention strategies.
 - Representatives of employers
 - Policy makers
 - Life sciences industry
 - Insurers

Rare Disease Advisory Panel

Background

- Rare Disease Roundtable (*September 11, 2013*)
 - Representatives included: Patients, patient advocacy organizations, researchers, government agencies, industry, and payers
 - Discussed a draft charter for the Rare Disease Advisory Panel
- Legislation envisions tailored panels for each rare disease study
- Draft charter constitutes a standing, overarching panel and allows for additional *ad hoc* advisory panels tailored to specific studies
- Charter has been endorsed by the Program Development Committee

Proposed Rare Disease Advisory Panel Charter

Purpose	Advise and provide recommendations on: • The conduct of patient-centered comparative clinical effectiveness research in rare diseases • Coordination and engagement with the rare disease research community (NOTE: The panel will not serve in an official decision-making capacity.)
Membership Term	Two-year staggered terms (maximum two terms)
Composition	• 12 to 15 members • No fewer than 33 percent to be persons who are rare disease patients, caregivers, or representatives of rare disease advocacy organizations • The remainder to include representation by broad array of stakeholders consistent with legislation
Structure: <i>Ad Hoc Panels</i>	The Panel will assist PCORI in identifying experts to serve on condition-specific <i>ad hoc</i> advisory panels to: • Assist in evaluating, designing, and conducting PCORI-funded research • Determine the relative value and feasibility of conducting the research study

Board Vote:

Rare Disease Advisory Panel Charter

Call for a Motion to:

- **Approve** the charter for the Rare Disease Advisory Panel

Call for the Motion to Be Seconded:

- **Second** the Motion
 - If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion

Vote:

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

Timeline and Next Steps

- With charter approval, PCORI will open in the coming months an application process for the panels
- First Advisory Panel meetings in Spring 2014