



Agenda

Advisory Panel on Rare Disease

Spring 2014 Meeting

April 30, 2014

**Hilton Alexandria Mark Center Hotel
5000 Seminary Rd
Alexandria, Virginia**

Wednesday, April 30, 2014

- 8:30 AM Welcome and Plans for the Day**
Joe V. Selby, MD, MPH, Executive Director, PCORI
- 8:35 AM Conflict of Interest Disclosures**
Joe V. Selby, MD, MPH, Executive Director, PCORI
- 8:45 AM Roles and Goals of the Advisory Panel on Rare Disease**
Bryan Luce, PhD, MBA, Chief Science Officer, PCORI
- 9:15 AM PCORI's Rare Disease Roundtable Report**
Greg Martin, Deputy Director, Stakeholder Engagement, PCORI
- 9:45 AM Break**
- 10:00 AM PCORI's Rare Disease Portfolio and Plans**
David Hickam, MD, Program Director, Clinical Effectiveness Research, PCORI
Rachael Fleurence, PhD, Program Director, CER Methods and Infrastructure, PCORI
- 12:00 PM Lunch**
- 1:00 PM – 4:15 PM Open Discussions**
- Discussion 1 (1:00–2:15 PM)**
Moderated by Bryan Luce, PhD, MBA, Chief Science Officer, PCORI
Topic 1: Provide input to PCORI on research needs of the rare diseases community and on specific issues and concerns in conducting research on rare diseases
Topic 2: Advise other PCORI committees and panels to ensure the unique considerations of rare disease are addressed
- Discussion 2 (2:15–4:15 PM)**
Moderated by Jean Slutsky, PA, MSPH, Chief Engagement and Dissemination Officer, PCORI
Topic 1: Provide ongoing feedback and advice on evaluating and disseminating PCORI's research portfolio on rare diseases
- 3:00 PM Break**

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3:15 PM

Discussion 2

Topic 2: Consider study findings and advise on targets and strategies for PCORI dissemination efforts

Topic 3: Identify opportunities for collaboration with existing international, federal, public and private entities doing similar work in the rare disease space

4:15 PM

Organizational Issues

Bryan Luce, PhD, MBA, Chief Science Officer, PCORI

5:00 PM

Post-Event Survey

5:15 PM

Recap and Next Steps

Bryan Luce, PhD, MBA, Chief Science Officer, PCORI

5:30 PM

Adjourn