



## 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**

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

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| <b>3:15 PM</b> | <b>Discussion 2</b><br><i>Topic 2: Consider study findings and advise on targets and strategies for PCORI dissemination efforts</i><br><i>Topic 3: Identify opportunities for collaboration with existing international, federal, public and private entities doing similar work in the rare disease space</i> |
| <b>4:15 PM</b> | <b>Organizational Issues</b><br>Bryan Luce, PhD, MBA, Chief Science Officer, PCORI   |
| <b>5:00 PM</b> | <b>Post-Event Survey</b>   |
| <b>5:15 PM</b> | <b>Recap and Next Steps</b><br>Bryan Luce, PhD, MBA, Chief Science Officer, PCORI  |
| <b>5:30 PM</b> | <b>Adjourn</b>   |