



## Agenda

**Advisory Panel on Rare  
Disease Fall 2015 Meeting**

**Friday, October 30, 2015**

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|-----------------|---|
| <b>8:30 AM</b>  | <b>Welcome and Plans for the Day</b><br>Hal Sox, MD, Chief Science Officer, PCORI<br>Vincent Del Gaizo, Co-Chair, Advisory Panel on Rare Disease, PCORI   |
| <b>8:45 AM</b>  | <b>Final PCORI Guidance on PCOR for Rare Diseases</b><br>Danielle Whicher, PhD, MHS, Program Officer, Clinical Effectiveness Research, PCORI  |
| <b>9:00 AM</b>  | <b>A Randomized Controlled Trial of Anterior Versus Posterior Entry Site for Cerebrospinal Fluid Shunt Insertion: Current Progress and Lessons Learned</b><br>William E. Whitehead, MD, MPH, Division of Pediatric Neurosurgery, Texas Children's Hospital, Baylor College of Medicine  |
| <b>9:45 AM</b>  | <b>Follow up Guidance to the Rare Disease Landscape Review</b><br>Parag Aggarwal, PhD, Senior Program Officer, Addressing Disparities, PCORI  |
| <b>10:15 AM</b> | <b>Break</b>  |
| <b>10:30 AM</b> | <b>Guidance for Rare Disease Research Breakout Groups</b> <ul style="list-style-type: none"><li>• <b>Human Subjects</b><br/>Patricia Furlong, Member, Advisory Panel on Rare Disease, PCORI<br/>Beth Kosiak, Program Officer, Improving Healthcare Systems, PCORI</li><li>• <b>Research Prioritization</b><br/>Marilyn Bull, MD, FAAP, Member, Advisory Panel on Rare Disease, PCORI<br/>Parag Aggarwal, PhD, Senior Program Officer, Addressing Disparities, PCORI</li><li>• <b>Challenges with Producing Reliable Evidence for Rare Diseases</b><br/>Naomi Aronson, PhD, Methodology Committee, PCORI<br/>Danielle Whicher, PhD, MHS, Program Officer, Clinical Effectiveness Research, PCORI</li></ul> |
| <b>12:15 PM</b> | <b>Lunch</b>  |
| <b>1:15 PM</b>  | <b>Reports from Breakout Groups</b><br>Patricia Furlong, Member, Advisory Panel on Rare Disease, PCORI<br>Marilyn Bull, MD, FAAP, Member, Advisory Panel on Rare Disease, PCORI<br>Naomi Aronson, PhD, Member, Methodology Committee, PCORI   |

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**2:15 PM**

**Update on PCORI's Rare Disease Portfolio**

Heather Edwards, PhD, MPH, MBA, Program Officer, Strategic Portfolio Analysis, PCORI

Mary Kay Margolis, MPH, MHA, Senior Program Officer, Evaluation and Analysis, PCORI

Vadim Y. Gershteyn, MPH, Program Associate, Evaluation and Analysis PCORI

**2:45 PM**

**Break**

**3:00 PM**

**PCORnet Update**

Maryam Zirkle, MD, MS, MA, Program Officer, CER Methods and Infrastructure, PCORI

**3:30 PM**

**Recap and Next Steps**

Vincent Del Gaizo, Co-Chair, Advisory Panel on Rare Disease, PCORI

Danielle Whicher, PhD, MHS, Program Officer, Clinical Effectiveness Research, PCORI

Parag Aggarwal, PhD, Senior Program Officer, Addressing Disparities, PCORI

**3:45 PM**

**Adjourn**