



Agenda

Advisory Panel on Rare Disease Fall 2015 Meeting

Friday, October 30, 2015

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