



Advisory Panel on Rare Disease Spring 2021 Meeting Agenda

May 14, 2021 | 1:00 – 5:00 PM ET

TIME	AGENDA ITEM	PRESENTERS
1:00 PM 10 minutes	Welcome, Introductions, and Setting the Stage	Scott Berns, Doug Lindsay
1:10 PM 5 minutes	Introduction of RDAP PCORI staff, RDAP Program Staff Update, Upcoming Activities	Carly Khan, Nora McGhee
1:15 PM 55 minutes	Rare Disease Research Awards: Study Highlights and Discussion	Penny Mohr, Jason Gerson
	<u>Project Title: Comparative Effectiveness of Palliative Surgery vs. Additional Anti-seizure medications for Lennox-Gastaut Syndrome</u> (1:25 - 1:30 pm)	Anne Berg, Sandi Lam, Tracy Dixon-Salazar
	<u>Project Title: Preserving Kidney Function in Children with Chronic Kidney Disease (PRESERVE)</u> (1:30 - 1:35 pm)	Michelle Denburg, Aneta Jovanovska, Rebecca Levondosky
	<u>Project Title: Utilizing PCORnet to Support Transition from Pediatric to Adult Centered Care and Reduce Gaps in Recommended Care in Patients with Congenital Heart Disease</u> (1:35 – 1:40 pm)	Tom Carton, Anitha John, Scott Leezer
	<u>Project Title: Comparative Effectiveness Research for Neuroendocrine Tumors (CER-NET)</u> (1:40 – 1:45 pm)	Michael O'Rorke, Josh Mailman
	<u>Project Title: Development of methods to improve identification of patients with rare or complex diseases</u> (1:45-1:50 pm)	Daniel Herman, Jinbo Chen
	Discussion (1:50 – 2:10 pm)	
2:10 PM 15 minutes	BREAK	
2:25 PM 45 minutes	Engagement Awards and Rare Disease Organizations: An Update and Project Highlights	Karen Martin Jennifer Canvasser

3:10 PM 30 minutes	Identifying our National Priorities: Relevance for Rare Disease Populations	Laura Lyman Rodriguez
3:40 PM 15 minutes	An Update on the Cost-Data Provision	Andrew Hu
3:55 PM 15 minutes	BREAK	
4:10 PM 30 minutes	COVID Connect: PCORI's Response to the COVID-19 Pandemic	Claudia Grossmann
4:40 PM 10 minutes	Acknowledgments and Recap	Scott Berns, Doug Lindsay
4:50 PM 10 minutes	Farewell to Departing Members	Doug Lindsay
5:00 PM	Adjourn	