



Agenda

Advisory Panel on
Rare Disease
In-Person Meeting

September 27th, 2017

Wednesday, September 27th, 2017
PCORI Offices

	9:00 AM	Welcome and Setting the Stage
		Matt Cheung, PhD, RPh, Chair
		Vincent Del Gaizo, Co-Chair
	9:30 AM	International Rare Diseases Research Consortium (IRDiRC) and Patient-Centered Outcome Measures
		Thomas Morel, MSc
		Research Fellow, <i>KU Leuven</i>
	10:00 AM	Core Outcome Set for Pediatric Rare Disease
		Gyasi Moscou-Jackson, PhD, MHS, RN
		Program Officer, <i>Healthcare Delivery and Disparities Research</i>
	11:30 AM	Break
	11:45 AM	Developing PCORI Informational Resources to Better Serve the Rare Disease Community
		Parag Aggarwal, PhD
		Senior Program Officer, <i>Healthcare Delivery and Disparities Research</i>
		William Silberg
		Director, <i>Communications</i>
	12:30 PM	Lunch
	1:15 PM	Presentation Materials for PCORI's Rare Disease Portfolio
		Parag Aggarwal, PhD
		Senior Program Officer, <i>Healthcare Delivery and Disparities Research</i>
	1:45 PM	Break
	2:00 PM	Case Study: PCORI Rare Disease Funded Study in Urea Cycle Disorders
		Nicholas Ah Mew, MD,
		Medical Geneticist, <i>Children's National</i>
	3:00 PM	Closing and Next Steps
		Matt Cheung, PhD, RPh, Chair
		Vincent Del Gaizo, Co-Chair
	3:30 PM	Adjourn