



## Agenda

Advisory Panel on  
Rare Disease  
In-Person Meeting

September 27<sup>th</sup>, 2017

Wednesday, September 27<sup>th</sup>, 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, *KU Leuven*
- 10:00 AM Core Outcome Set for Pediatric Rare Disease**  
Gyasi Moscou-Jackson, PhD, MHS, RN  
Program Officer, *Healthcare Delivery and Disparities Research*
- 11:30 AM Break**
- 11:45 AM Developing PCORI Informational Resources to Better Serve the Rare Disease Community**  
Parag Aggarwal, PhD  
Senior Program Officer, *Healthcare Delivery and Disparities Research*  
William Silberg  
Director, *Communications*
- 12:30 PM Lunch**
- 1:15 PM Presentation Materials for PCORI's Rare Disease Portfolio**  
Parag Aggarwal, PhD  
Senior Program Officer, *Healthcare Delivery and Disparities Research*
- 1:45 PM Break**
- 2:00 PM Case Study: PCORI Rare Disease Funded Study in Urea Cycle Disorders**  
Nicholas Ah Mew, MD,  
Medical Geneticist, *Children's National*
- 3:00 PM Closing and Next Steps**  
Matt Cheung, PhD, RPh, Chair  
Vincent Del Gaizo, Co-Chair
- 3:30 PM Adjourn**