



Agenda

**Advisory Panel Winter
2015 Meeting**

**Advisory Panel on Rare
Disease**

January 13, 2015

9:30 AM	Welcome and Plans for the Day Bryan Luce, PhD, MBA, Chief Science Officer, PCORI Marshall L. Summar, MD, Chair, Advisory Panel on Rare Disease, PCORI
9:45 AM	Analysis of PCORI Review of Applications on Rare Diseases Laura Forsythe, PhD, MPH, Senior Program Officer, PCORI Vadim Y. Gershteyn, MPH, Program Associate, PCORI
10:45 PM	Break
11:00 AM	Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options Topic Prioritization Topic: "Genetic Testing for Rare Diseases: Compare the effectiveness of genetic testing for select rare diseases in terms of patient care, treatment choices, and relevant clinical and patient-centered outcomes?" Topic Experts: Marshall L. Summar, MD, Chair, Advisory Panel on Rare Disease, PCORI Division Chief, Genetics and Metabolism, Margaret O'Malley Chair in Genetic Medicine, Medical Director, Clinical Research Center, and Vice-Chair External Affairs, Children's National Medical Center Professor of Pediatrics, George Washington Medical College Uday Deshmukh, Member, Advisory Panel on Rare Disease, PCORI Senior Medical Director of Clinical Operations, Florida Blue
12:30 PM	Lunch (Crystal Ballroom Salon A)
1:30 PM	Clinical Trials in Rare Diseases: <i>Starting from Scratch Even with Limited Resources</i> Jason Connor, PhD, Member, Advisory Panel on Clinical Trials Director and Senior Statistical Scientist, Berry Consultants
2:30 PM	Ad Hoc Advisory Panels on Rare Disease Bryan Luce, PhD, MBA, Chief Science Officer, PCORI Emma Djabali, Project Assistant, Office of the Chief Science Officer, PCORI
3:15 PM	Break
3:30 PM	Update about Collaboration with CTAP Bryan Luce, PhD, MBA, Chief Science Officer, PCORI
4:00 PM	Compensating Patient Partners in Research Suzanne Schrandt, JD, Deputy Director, Patient Engagement, PCORI
4:45 PM	Recap and Next Steps Bryan Luce, PhD, MBA, Chief Science Officer, PCORI

Marshall L. Summar, MD, Chair, Advisory Panel on Rare Disease, PCORI

5:00 PM

Adjourn