

## Conference on Clinical Research for Rare Diseases Thursday November 3, 2016

<b>Morning Session</b>	<b>Lecture Title/Event</b>	<b>Summary</b>	<b>Suggested Speakers</b>
7:00 – 8:00	<i>Breakfast, Registration, and Poster Set-up</i>		
8:00 – 8:10	<i>Welcome and Introduction</i>	Review agenda, introductions	Petra Kaufmann, MD, MSc Peter Merkel, MD, MPH
8:10 – 8:30	<i>“Challenges and solutions to conducting clinical research in rare diseases”</i>	Outline of major issues with additional intent of setting up the day	Peter Merkel, MD, MPH
8:30 – 9:15	<i>“Clinical trial design issues and options for study of rare diseases”</i>	Studies of small sizes; trial design (open-label, multicenter, N-of1), etc	Jeffrey Krischer, PhD
9:15 – 9:45	<i>“Systems biology, genetics, and big data in the study of rare disease”</i>	Applying exciting and powerful new techniques in integrative biology to the study of rare diseases	Matthias Kretzler, MD
9:45 – 10:00	<i>Break</i>		
10:00 – 11:00	<i>How to work with Industry and the FDA for rare diseases research</i>	Representatives from Industry and the FDA.	Richard Moscicki, MD David Whiteman, MD
11:00 – 11:30	<i>Importance of Investigator and Patient Advocacy Group Collaborations</i>	2 Investigators, and 2 CPAG partners	Michio Hirano, MD Ellyn Kodroff Charles Mohan Marc Rothenberg, MD, PhD Mary Jo Strober
11:30 – 12:30	<i>Highlighting novel/award-winning abstracts</i>	Poster presentations with panel input/critique	Shubhasree Banerjee, MD Elizabeth Bhoj, MD, PhD Dong Li, PhD Mei-Sing Ong, PhD
12:30 – 12:45	<i>Buffet lunch pick-up</i>		
12:45 – 2:00	<i>Poster session through lunch</i>		
<b>Afternoon Session</b>			
2:00 – 3:15	<i>Breakout session 1</i>	<ul style="list-style-type: none"> <li>• Career development and mentoring <b>OR</b></li> <li>• Novel approaches to rare diseases trial design</li> </ul>	All breakouts repeated in session 2 with same speakers
3:15 – 3:30	<i>Break</i>		
3:30 – 4:45	<i>Breakout session 2</i>	<ul style="list-style-type: none"> <li>• Career development and mentoring <b>OR</b></li> <li>• Novel approaches to rare diseases trial design</li> </ul>	
5:00 – 5:30	<i>Critical evaluation of day’s program</i>	Written (anonymous) evaluation form with comments; open discussion	Attendees and Organizing Committee
<b>Evening Session</b>			
5:30 – 7:00	<i>Wine and cheese reception and additional poster discussion</i>	Informal session Trainee networking	All meeting attendees