

Conference on Clinical Research for Rare Diseases Monday November 19, 2018

Morning Session	Lecture Title/Event	Summary	Suggested Speakers
7:00 – 8:00	<i>Breakfast, Registration, and Poster Set-up</i>		
8:00 – 8:10	<i>Welcome and Introduction</i>	Review agenda, introductions	Christopher P. Austin, MD 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>“Considerations for the use of biomarkers in the study of rare diseases”</i>	Discussing the unique issues of utilizing biomarkers as study outcomes in research in rare diseases	Kathleen Donohue, 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 biomedical industry and the FDA outline the many complex issues involved in such collaborations	Lucas Kempf, MD Mitchell Goldman, MD, PhD
11:00 – 11:30	<i>Importance of investigator and patient advocacy group (PAG) Collaborations</i>	2 Investigators, and 2 CPAG partners discuss these critical interactions and partnerships	Tracy Hart, Alice McConnell, Brendan Lee, MD, PhD Jean-Baptiste Rouillet, PhD
11:30 – 12:30	<i>Highlighting novel/award-winning abstracts</i>	Poster presentations with panel input/critique	
12:30 – 12:45	<i>Buffet lunch pick-up</i>		
12:45 – 2:00	<i>Poster session through lunch</i>		
Afternoon Session			
2:00 – 3:15	<i>Breakout session 1</i>	<ul style="list-style-type: none"> • Career development and mentoring OR • Practical logistics of conducting research in rare disease 	
3:15 – 3:30	<i>Break</i>		
3:30 – 4:45	<i>Breakout session 2</i>	<ul style="list-style-type: none"> • Career development and mentoring OR • Practical logistics of conducting research in rare disease 	All breakouts repeated in session 2 with same speakers
5:00 – 5:30	<i>Critical evaluation of day’s program</i>	Written (anonymous) evaluation form with comments; open discussion	Attendees and Organizing Committee
Evening Session			
5:30 – 7:00	<i>Wine and cheese reception and additional poster discussion</i>	Informal session Trainee networking	All meeting attendees