Welcome and Introduction

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CCRRD Sponsors

• This conference is a program of

➢ The Rare Diseases Clinical Research Network (RDCRN)
Thanks to Our Financial Supporters

- National Center for Advancing Translational Sciences
- National Institute of Arthritis and Musculoskeletal and Skin Disease
- Primary Ciliary Dyskinesia Foundation
- Vasculitis Foundation
- Vasculitis Clinical Research Consortium
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Housekeeping Items

• **Meals**
  - Breakfast—now
  - Working Lunch/Poster Session—in the Atrium
  - Caffeine, fluids, snacks—continuous!
  - Breaks—exist
PLEASE put your cell phones and pagers on vibration or silent modes

Better yet, turn them off and actually enjoy the meeting!
Today’s Program

- **Target audience:**
  - Trainees and junior faculty engaged in clinical investigation into rare diseases
  - Any others engaged in research in rare diseases
  - List of attendees included in the binder

- **Lectures/Panels:**
  - Save up questions (write them down)
  - Goal is to have interchange

- **Poster session**

- **Workshops**

- **Reception**
Evaluation Forms

• Please complete after each talk
• Your comments are quite important and will help determine content of future conferences
• Please return the completed forms to the basket on the registration desk (or we will collect them last session)
“Challenges and solutions to conducting clinical research in rare diseases”

Peter A. Merkel, MD, MPH
University of Pennsylvania
“Clinical trial design issues and options for study of rare diseases”

Jeffrey P. Krischer, PhD
*University of South Florida*
“Considerations for the use of biomarkers in the study of 
rare diseases”

Kathleen Donohue, MD
Clinical Team Lead
Division of Gastroenterology & Inborn Errors of Metabolism
US Food and Drug Administration
“How to work with Industry and the FDA for rare diseases research”

Mitchell Goldman, MD, PhD
AstraZeneca

Lucas Kempf, MD
U.S. Food and Drug Administration

Bruce Trapnell, MD
Cincinnati Children’s Hospital Medical Center
“Importance of Investigator and Patient Advocacy Group Collaborations”

Tracy Hart
*Osteogenesis Imperfecta Foundation*

Brendan Lee, MD, PhD
*Baylor College of Medicine*

Alice McConnell
*Speragen, Inc.*

Jean-Baptiste Roullet, PhD
*Washington State University*
Steve Groft honorary sponsorship of the CCRRD meeting on behalf of CPAG:

“Award winning abstracts”
“Award winning abstracts”

Onur Cil, MD, PhD
Thomas Greuter, MD
Kaitlin Quinn, MD
Mario Saporta, MD, PhD
Poster Session (and Lunch)

• Poster Board number—see list in folder
  – Downstairs in the Atrium
  – 12:30 – 2:00 PM

• Poster session
  – Please grab some food and stand by your poster for most of the session
  – Take down poster at end of night or the poster will be discarded
  – Can also view posters during breaks

• Senior investigators and NIH program staff will be viewing your posters—good time for feedback, etc.
Afternoon Breakout Sessions

• Parallel small-group breakouts

• 2 sessions for each of 2 breakouts
  – Career development and mentoring
  – Practical logistics of conducting research in rare disease

• Each breakout will be repeated so that all attendees will take part in a breakout on both topics

• Attendees assigned to break-out sessions
  – See the back of your name tag for room assignments
  – Rooms are next to each other, after the first session move to the room next door for the second session
Breakout 1: Career Development and Mentoring

• Panel discussions by senior faculty mentors and trainees/junior faculty

• Focus on issues related to trainees studying rare diseases
Breakout 2: Practical logistics of conducting research in rare disease

• Short presentations by principal investigators and subsequent discussion

• Focus on examples of successful approaches and unexpected challenges to conducting research in rare diseases
Wine and cheese reception

• Informal setting for networking

• All attendees encouraged to join the reception