



RDCRN Conference on Clinical Research for Rare Diseases 2016

Washington, DC
November 3, 2016

Welcome and Introduction

Peter A. Merkel, MD, MPH
University of Pennsylvania

CCRRD Sponsors

- **This conference is a program of**
 - **The Rare Diseases Clinical Research Network (RDCRN)**
- **With financial support from:**
 - The NCATS
 - NHLBI, NIAMS, NINDS, PAG: PCD foundation



Thanks to Our Financial Supporters

National Institutes of Health



National Center
for Advancing
Translational Sciences

- National Center for Advancing Translational Sciences (NCATS)



National Heart, Lung,
and Blood Institute

- National Heart, Lung, and Blood Institute (NHLBI)



National Institute of
Arthritis and Musculoskeletal
and Skin Diseases

- National Institute of Arthritis and Musculoskeletal and Skin Disease (NIAMS)



National Institute of
Neurological Disorders
and Stroke

- National Institute of Neurological Disorders and Stroke (NINDS)

Patient Advocacy Group Support



- Primary Ciliary Dyskinesia (PCD) Foundation



Thanks to many folks

- **Organizing Committee**

- Anne Comi, MD
- Robert J. Desnick, PhD, MD
- Matthias Kretzler, MD
- Jeffrey P. Krischer, PhD
- Michelle Manion
- Carol A. McAlear, MA
- Peter A. Merkel, MD, MPH, Co-Chair
- Marc E. Rothenberg, MD, PhD
- Michael Shy, MD, Co-Chair
- Vernon Reid Sutton, MD
- Bruce C. Trapnell, MD
- Chester B. Whitely, PhD, MD



Thanks to many folks

- **NIH Liaisons**
 - Petra Kaufmann, MD, MSc
 - Rashmi Gopal-Srivastava, PhD
 - David Eckstein, PhD
- **Data Management Coordinating Center (DMCC)**
 - Jeffrey P. Krischer, PhD,
 - Jennifer Harris, MSPH
 - Joseph L. Gomes
- **University of Pennsylvania**
 - Carol McAlear, MA
 - Kathleen Ryan

Thanks to many folks...



...but especially Carol McAlear!

PLEASE put your cell phones and
pagers on vibration or silent
modes

**Better yet, turn them off and
actually enjoy the meeting!**



Housekeeping Items

- **Calories, fluids, and stimulants**
 - **Breakfast: Now**
 - **Lunch:**
 - **Washington Room**
 - **Please rapidly procure food and drink and proceed into the poster room**
 - **Perhaps let the poster presenters go first**
 - **Caffeine, fluids, snacks: Continuous!**
 - **Breaks: Yes**
 - **Wine & Cheese Reception: Poster room**



Travel Awards

- Travel award recipients **MUST** be sure to receive reimbursement envelope from registration desk-*do not leave without one*
- You were emailed the forms and they will be re-sent on November 4, 2016
- Follow the directions completely
- Monday, November 21, 2016: Strict deadline for returning reimbursement form and all necessary documents; *we are not kidding!*



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**



***“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



***“Systems biology, genetics, and big data in
the study of rare disease”***

Matthias Kretzler, MD
University of Michigan



“How to work with Industry and the FDA for rare diseases research”

Richard Moscicki, MD
US Food and Drug Administration

David Whiteman, MD
Shire Pharmaceuticals, Inc.



***“Importance of Investigator and Patient
Advocacy Group Collaborations”***

**Charles A. Mohan, Jr.,
*United Mitochondrial Disease Foundation***

**Michio Hirano, MD
*Columbia University Medical Center***

**Ellyn Kodroff, Mary Jo Strobel
*Campaign Urging Research for Eosinophilic Disease***

**Marc E. Rothenberg, MD, PhD
*Cincinnati Children’s Hospital Medical Center***



“Award winning abstracts”

Shubhasree Banerjee, MD

Elizabeth Bhoj, MD, PhD

Dong Li, PhD

Mei-Sing Ong, PhD



Poster Session (and Lunch)

- **Set up time: last night, now (breaks)**
- **Poster-board number—see list**
- **Poster session**
 - Please grab some food and stand by your poster for most of the session
 - Also use the Wine and Cheese Reception time to review posters
 - 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 3 breakouts**
- **Each breakout will be repeated so that all attendees will take part in a breakout on both topics**
- **All attendees are pre-assigned to groups for both sessions (look on the back of your nametag)**



Afternoon Breakout Sessions

- Please attend you assigned group or you will mess up the system!

– Hoover: Breakout 1A/2A



Career Development and Mentoring

– Coolidge: Breakout 1B/2B

– Harding: Breakout 1C/2C



Novel Approaches to Rare Diseases Trial Design

– The rooms are all beside each other.



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**
- **Content is for both mentees and mentors**



Breakout 2: Novel Approaches to Rare Disease Trial Design

- **Short presentations by principal investigators and subsequent discussion**
- **Focus on examples of successful approaches and unexpected challenges to conducting research in rare diseases**



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)



Wine and cheese reception

- **Informal setting for networking**
- **All attendees encouraged to join the reception**
- **Mingle among the posters**





RDCRN Conference on Clinical Research for Rare Diseases 2016

Washington, DC
November 3, 2016