Welcome and Introduction

Peter A. Merkel, MD, MPH
University of Pennsylvania
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 (NCATS)
- National Heart, Lung, and Blood Institute (NHLBI)
- National Institute of Arthritis and Musculoskeletal and Skin Disease (NIAMS)
- National Institute of Neurological Disorders and Stroke (NINDS)

Patient Advocacy Group Support

- Primary Ciliary Dyskinesia (PCD) Foundation
Thanks to many folks

- **Organizing Committee**
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  - 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
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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 resent 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 Cheest 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 your assigned group or you will mess up the system!
  – Hoover: Breakout 1A/2A
  – Coolidge: Breakout 1B/2B
  – Harding: Breakout 1C/2C
  – 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