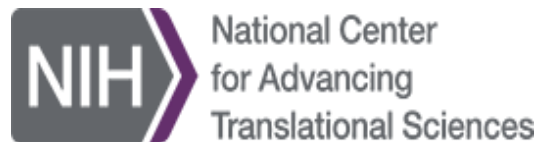




# 5th Conference on Clinical Research for Rare Diseases

November 19, 2018 • Hilton Rockville Hotel & Executive Meeting Center • Rockville, MD



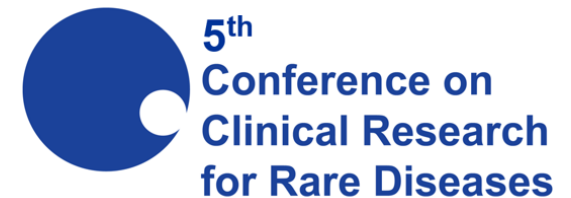
# Welcome and Introduction

**Peter A. Merkel, MD, MPH**  
**Professor of Medicine and Epidemiology**  
**University of Pennsylvania**  
**Principal Investigator**  
**Vasculitis Clinical Research Consortium**



# CCRRD Sponsors

- This conference is a program of
  - The Rare Diseases Clinical Research Network (RDCRN)



# Thanks to Our Financial Supporters



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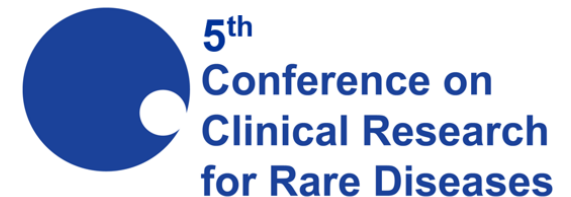
- Primary Ciliary Dyskinesia Foundation



- Vasculitis Foundation



- Vasculitis Clinical Research Consortium



# Thanks to many folks

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- Christopher P. Austin, MD
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## Data Management Coordinating Center (DMCC)

- Jeffrey P. Krischer, PhD
- Jennifer Harris, MSPH
- Joseph L. Gomes

## University of Pennsylvania

- Carol McAlear, MA

# 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.***

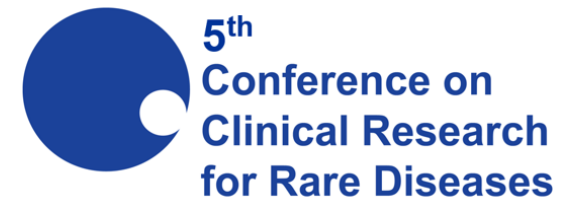
**Jean-Baptiste Rouillet, PhD**  
***Washington State University***





***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