

## Welcome to Day 1

- 9:45 a.m. – 9:55 a.m. **Welcome and Day 1 Introduction**  
Terry Watnick, M.D., Professor of Medicine, Joan B. and John H. Sadler Professor in Nephrology, and Principal Investigator, PKD RRC Coordinating Site, University of Maryland School of Medicine

## Session 1: Cutting-Edge Techniques or Models That Could Be Utilized for PKD Research

Session Chairs: Stefan Somlo, M.D., Yale University School of Medicine  
Luis Menezes, M.D., National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)

- 9:55 a.m. – 10:15 a.m. **Merging Novel Approaches in Genome and Phenome Science for PKD Research**  
Nancy Cox, Ph.D., Professor, Vanderbilt University Medical Center
- 10:15 a.m. – 10:20 a.m. *Questions*
- 10:20 a.m. – 10:40 a.m. **Vascularized Kidney Organoids: Integrating Stem Cell Differentiation with Bioprinting to Create Improved PKD Models**  
Jennifer Lewis, Sc.D., Hansjörg Wyss Professor, Harvard John A. Paulson School of Engineering and Applied Sciences and Wyss Institute
- 10:40 a.m. – 10:45 a.m. *Questions*
- 10:45 a.m. – 11:05 a.m. **A Human Kidney Organoid Platform for Autosomal Dominant PKD (ADPKD)**  
Andy McMahon, Ph.D., Professor, Keck School of Medicine of University of Southern California
- 11:05 a.m. – 11:10 a.m. *Questions*
- 11:10 a.m. – 11:30 a.m. **Cell Atlases as Roadmaps to Understanding Disease**  
Orit Rozenblatt-Rosen, Ph.D., Genentech
- 11:30 a.m. – 11:35 a.m. *Questions*

## 11:35 a.m. – 12:15 p.m. Trainee Short Communications

(5 trainee abstracts, 5-minute presentations with 3 minutes for questions each, 1–3 slides)

Session Chairs: Bernie Gitomer, Ph.D., University of Colorado Denver  
Takamitsu Saigusa, M.D., The University of Alabama at Birmingham

## 12:15 p.m. – 12:45 p.m. Lunch Break

## Challenge Sessions A and B—Concurrent

- 12:45 p.m. – 1:45 p.m. **Challenge A: Developing Biomarkers for Measuring PKD Disease Progression—Do We Know as Much as We Think We Do?**  
Session Chairs: Ron Perrone, M.D., Tufts Medical Center  
Erum Hartung, M.D., MTR, Children's Hospital of Pennsylvania
- 12:45 p.m. – 1:05 p.m. **Urinary Biomarkers in PKD: Potential Value and Link to Pathophysiology**  
Harald Mischak, Ph.D., Mosaiques Diagnostics and Professor, University of Glasgow
- 1:05 p.m. – 1:25 p.m. **The Long Road to Biomarkers of Polycystic Kidney Disease**  
Jon Klein, M.D., Ph.D., Professor, University of Louisville School of Medicine
- 1:25 p.m. – 1:45 p.m. **Panel Discussion:** Ron Perrone, Erum Hartung, Neera Dahl, Jon Klein, Ronak Lakhia, Max Liebau, Harald Mischak, and Kristen Nowak

12:45 p.m. – 1:45 p.m. **Challenge B: *The complexity of ADPKD pathogenesis in humans has made difficult the discovery of the intrinsic function of the polycystin protein complex. Current work supports a number of different signaling modalities for the polycystin complex, including functioning as an ion channel, a G protein-coupled receptor, and others. What tools are necessary to clarify how the polycystins function in signaling pathways?***

Session Chairs: Vishal Patel, M.D., Associate Professor, The University of Texas Southwestern Medical Center  
Rebecca Walker, DPhil., University of Maryland School of Medicine

12:45 p.m. – 1:05 p.m. **Defining How the Polycystins Prevent Cyst Formation: Are We Ready to Fill the Gap?**  
Alessandra Boletta, Ph.D., Group Leader, Ospedale San Raffaele

1:05 p.m. – 1:25 p.m. **Whom Do the Polycystins Talk to, What Do They Say, and How Can We Listen in?**  
Michael Caplan, M.D., Ph.D., Professor, Yale University School of Medicine

1:25 p.m. – 1:45 p.m. **Panel Discussion:** Vishal Patel, Rebecca Walker, Alessandra Boletta, Michael Caplan, Marcus Delling, Ken Hallows, Feng Qian, and Kurt Zimmerman

**1:45 p.m. – 2:00 p.m. Break**

### **Controversy Sessions A and B—Concurrent**

2:00 p.m. – 3:00 p.m. **Controversy A: *Both haploinsufficiency and secondary somatic mutations of the PKD genes have been reported clinically. Is the frequency of one dominant? Which should the field focus on for model and therapeutic development?***

Session Chairs: York Pei, M.D., Professor, University of Toronto  
Cynthia Sieben, Ph.D., Mayo Clinic

2:00 p.m. – 2:20 p.m. **Insufficient Polycystin Function Is the Principal Cause of ADPKD—Does It Matter How Many “Hits” Cause It?**  
Greg Germino, M.D., Deputy Director, Senior Investigator, NIDDK

2:20 p.m. – 2:40 p.m. **The Case for a Dosage/Threshold Model of Cyst Initiation in ADPKD**  
Peter Harris, Ph.D., Professor, Mayo Clinic

2:40 p.m. – 3:00 p.m. **Panel Discussion:** York Pei, Cynthia Sieben, Greg Germino, Peter Harris, Katharina Hopp, Matthew Lanktree, Hanna Rennert, and Chris Ward

2:00 p.m. – 3:00 p.m. **Controversy B: *Is PKD Only a Ciliopathy? Is It Time to Reassess the Role of Polycystin Function Outside the Cilia in Human Disease?***

Session Chairs: Barbara Ehrlich, Ph.D., Professor, Yale University School of Medicine  
Eryn Dixon, Ph.D., Washington University School of Medicine in St. Louis

2:00 p.m. – 2:20 p.m. **PKD Is a Ciliopathy**  
Greg Pazour, Ph.D., Professor, University of Massachusetts Medical School

2:20 p.m. – 2:40 p.m. **Evidence Supporting a Role of Polycystins Outside the Cilia**  
Michael Köttgen, M.D., Professor, University of Freiburg

2:40 p.m. – 3:00 p.m. **Panel Discussion:** Barbara Ehrlich, Eryn Dixon, Michael Köttgen, Karel Liem, Moe Mahjoub, Greg Pazour, and Brad Yoder

**3:00 p.m. Adjournment**

## Welcome to Day 2

### 10:00 a.m. – 10:05 a.m. **Day 2 Introduction**

Brad Yoder, Ph.D., Professor of Cell Biology, Endowed Chair in Biomedical Research

### 10:05 a.m. – 11:45 a.m. **Session 2: Biology and Function of the Polycystins**

Session Chairs: Dorien Peters, Ph.D., Professor, Leiden University Medical Center  
Whitney Besse, M.D., Assistant Professor, Yale University School of Medicine

### 10:05 a.m. – 10:25 a.m. **TBA**

Ke Dong, M.D., Yale University School of Medicine

### 10:25 a.m. – 10:30 a.m. *Questions*

### 10:30 a.m. – 10:50 a.m. **Potential Role of AMP-Activated Protein Kinase in Metabolic Reprogramming**

Greg Steinberg, Ph.D., Professor, McMaster University

### 10:50 a.m. – 10:55 a.m. *Questions*

### 10:55 a.m. – 11:15 a.m. **How Cilia Decipher cAMP**

Jeremy Reiter, M.D., Ph.D., Professor University of California San Francisco

### 11:15 a.m. – 11:20 a.m. *Questions*

### 11:20 a.m. – 11:40 a.m. **Understanding Polycystic Kidney Disease Through the Lens of the Electron Microscope**

Qiang Su, Ph.D., Westlake University

### 11:40 a.m. – 11:45 a.m. *Questions*

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### 12:15 p.m. – 1:15 pm **Trainee Short Communications**

(7 trainee abstracts, 5-minute presentations with 3 minutes for questions each, 1–3 slides)

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Takamitsu Saigusa, M.D., Associate Professor, The University of Alabama at Birmingham

### 1:15 p.m. – 2:30 p.m. **Challenges and Controversies Sessions Day 2**

Moderator: Stefan Somlo, M.D., Yale University School of Medicine

- All topic groups and panelists meet as one.
  - **Challenge Session A: *Developing Biomarkers for Measuring PKD Progression***  
Chairs: Ron Perrone and Erum Hartung
  - **Challenge Session B: *The complexity of ADPKD pathogenesis in humans***  
Chairs: Vishal Patel and Rebecca Walker
  - **Controversy Session A: *Haploinsufficiency vs secondary somatic mutations***  
Chairs: York Pei and Cynthia Sieben
  - **Controversy Session B: *Is PKD Only a Ciliopathy?***  
Chairs: Barbara Ehrlich and Eryn Dixon
- Sessions chairs for each topic group will present the distillation of the previous day's discussions with the support of the panelists from their respective groups for up to 8 minutes.
- Each topic presentation will be followed by a moderated discussion with the entire conference audience for 10 minutes.

**2:30 p.m. – 3:00 p.m. Community Feedback—Critical Needs**

Breakout sessions will be hosted by each of the consortium divisions and focused on feedback from the community on what their critical needs are for new models and tools.

**Breakout 1: *In Vitro* Models, Chair: Darren Wallace**

Moderators: Lisa Guay-Woodford and Alan Yu

**Breakout 2: Animal Models, Chair: Brad Yoder**

Moderators: Patricia Outeda-Garcia and Pamela Tran

**Breakout 3: Clinical Resources, Chair: Steve Seliger**

Moderators: John Parant and Owen Woodward

**3:00 p.m.**

**Adjournment**

**Thank you for attending our First Annual Virtual PKD-RRC Symposium!**