

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. – 12:10 p.m. Trainee Short Communications

(4 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

12:10 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. **TBA**
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. **Lessons from the Adhesion G Protein-Coupled Receptor Field for PKDs**

Demet Arac, Ph.D., Associate Professor, The University of Chicago

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*

11:45 a.m. – 12:30 p.m. **Lunch Break**

12:30 p.m. – 1:15 pm **Trainee Short Communications**

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

Session Chairs: Bernie Gitomer, Ph.D., Professor, University of Colorado Denver
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.
- 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.
- Each topic presentation will be followed by a moderated discussion with the entire conference audience.

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

Breakout 2: Animal Models, Chair: Brad Yoder

Breakout 3: Clinical Resources, Chair: Steve Seliger

3:00 p.m.

Adjournment

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