

# 2023 PKD RRC SYMPOSIUM SPEAKER LIST

Location: Zoom  
Date: September 26-27, 2023  
Time: 9:00 a.m. - 4:00 p.m. EDT  
Facilitator: Dr. Terry Watnick

## Day 1 – Session 1

### Speaker Name/Institution

**Shiaulou Yuan**, Ph.D., Assistant Professor of Medicine @ Cardiovascular Research Center Massachusetts General Hospital and Harvard Medical School

**Robby Van Sciver**, Ph.D., Postdoctoral Fellow @ Emory University School of Medicine

## Ciliary Signaling

### Speaker Biosketch/introduction

Dr. Shiaulou Yuan is Assistant Professor of Medicine at the Cardiovascular Research center at Massachusetts General Hospital and Harvard Medical School. His research focuses on understanding the cellular mechanisms that shape the developing heart. The title of his talk is “Left, right and center: cilia and polycystin-2 as mechanotransducers during left-right axis development”.

Robert Van Sciver is a post-doctoral fellow in the Caspary lab at Emory University School of Medicine. He joined Dr. Tamara Caspary’s lab in 2019 as an IRACDA postdoctoral fellow, where he carved out a project investigating the role of ARL13B in kidney cysts. The title of his talk is “ARL13B cilia localization and GEF activity in polycystic kidney disease”

### Presentation Title

“Left, right and center: cilia and polycystin-2 as mechanotransducers during left-right axis development”

ARL13B cilia localization and GEF activity in polycystic kidney disease

## Day 1 – Session 2

## Is Gene Therapy for PKD on the Horizon, etc.

### Speaker Name/Institution

**Angela Lek, Ph.D.,**  
VP of research, Muscular  
Dystrophy Association

### Speaker Biosketch/introduction

Dr. Lek is the VP of Research at the Muscular Dystrophy Association. She is a PhD-trained scientist who has worked on disease modeling and therapy development for muscular dystrophies. Her current position involves working with stakeholders to achieve translation of genetic therapies. She also serves as the scientific lead for MDA's in-house gene therapy development program for ultra-rare neuromuscular disorders. The title of her talk is "Learnings from neuromuscular disease gene therapies"

### Presentation Title

Learnings from  
neuromuscular disease  
gene therapies

**Jason Tisdale, M.D.**  
Director, Trans-NIH Intramural Sickle  
Cell Program  
National Heart, Lung, and Blood  
Institute  
National Institutes of Health Branch  
Chief, Cellular and  
Molecular Therapeutics Branch

Dr. Tisdale is the Branch Chief for the Cellular and Molecular Therapeutics Branch and he also serves as the Director of the Trans-NIH Intramural Sickle Cell Program at the National Heart, Lung, and Blood Institute. Dr. Tisdale's work centers on sickle cell disease specifically developing curative strategies for sickle cell disease through transplantation of allogeneic or genetically modified autologous bone marrow stem cells. The title of his talk is "The long and winding road to molecular cures for the first molecular disease".

The long and winding road  
to molecular cures for the  
first molecular disease

**Matthew Wilson, M.D., Ph.D.,** Professor  
Department of Medicine  
Division of Nephrology and  
Hypertension  
Vanderbilt University Medical Center

Dr. Wilson is Professor of Medicine in the Division of Nephrology and Hypertension at Vanderbilt University Medical Center. He is a physician-scientist nephrologist whose current research is focused on developing cell and gene therapy for kidney disease, The title of his talk is Targeting the kidneys with gene therapy

Targeting the kidneys with  
gene therapy

## Day 1 Session 3

### Speaker/Institution

Alan Yu, M.B., B. Chir., Professor of Medicine, The University of Kansas Medical Center

## Next Steps in identifying PKD therapies: Brainstorming

### Speaker BioSketch/Introduction

Dr. Yu is the Harry Statland Professor of Medicine, Director of the Jared Grantham Kidney Institute, and Director of the Division of Nephrology and Hypertension at the University of Kansas Medical Center. He has a background in renal physiology research and clinical research in PKD. He serves as the principal investigator for CRISP, the longest running cohort study of PKD, and as co-chair of the Clinical Subcommittee for the PKD RRC. The title of his talk is “Therapy for PKD – Quo vadis?”

### Presentation Title

Therapy for PKD – Quo vadis?

## Day 1 – Session 4

### Speaker/Institution

Zhongwei Li, Ph.D.,  
Assistant Professor  
Of Medicine, Stem Cell Biology  
and Regenerative Medicine,  
Keck school of Medicine

Kirsten Brennand, Ph.D.,  
Professor of Psychiatry, Yale  
University School of Medicine

## Tubuloids and Organoids

### Speaker Biosketch/Introduction

Dr. Li is Assistant Professor of Medicine, Stem Cell Biology and Regenerative Medicine at the Keck school of Medicine. His lab uses state of the art approaches to develop novel strategies for kidney regeneration and to establish platforms for kidney disease modeling and drug discovery. The title of his talk is “Novel stem cell tools to model polycystic kidney disease”

### Presentation Title

“Novel stem cell tools to model polycystic kidney disease”

### Speaker Biosketch/Introduction

Kristen Brennand is the Elizabeth Mears and House Jameson Professor of Psychiatry and Professor of Genetics at Yale University School of Medicine. Her current research combines expertise in human stem cell models, genomic engineering, and neuroscience to identify the mechanisms that underlie brain development, traits, and disease. The title of her talk is “Using Stem Cells to Explore the Genetics Underlying Brain Disease”.

### Presentation Title

Using Stem Cells to Explore the Genetics Underlying Brain Disease.

## Day 2 – Session 5

## New techniques: Applications to Kidney Disease

### Speaker/Institution

**Jason Gleghorn**, Ph.D., Associate Professor, Department of Biomedical Engineering, University of Delaware

### Speaker Biosketch/Introduction

Dr. Jason Gleghorn is Associate Professor in the Department of Biomedical Engineering at the University of Delaware. He leads an interdisciplinary laboratory focused on developing disease models and therapeutics that integrate innate and adaptive immune responses and drug transport with applications in women's health, maternal-fetal health, and in congenital birth defects. The title of his talk is "Closing the tech transfer gap: democratizable modular 3D microphysiological tissue models".

### Presentation Title

Closing the tech transfer gap: democratizable modular 3D microphysiological tissue models

### Speaker/Institution

**Tarek M. Ashkar (El-Achkar)**, M.D., FASN, Terence P. Kahn Professor of Nephrology, Professor of Medicine, Adjunct Professor of Physiology, Anatomy and Cell Biology, Indiana University and the Indianapolis VA Medical Center

### Speaker Biosketch/Introduction

Dr. Ashkar is the Terrence P. Kahn Professor of Nephrology at Indiana University. In addition to studying the biology of the protein uromodulin, he is an investigator in the Kidney Precision Medicine Project (KPMP) and the Human BioMolecular Atlas Program (HuBMAP). The aim of this work to unravel the cellular and molecular pathophysiology of kidney disease through integrating multimodal Omics and imaging approaches. The title of his talk is "Spatial analysis at cellular resolution to study human kidney disease"

### Presentation Title

Spatial analysis at cellular resolution to study human kidney disease

### Speaker/Institution

**KotDaji Ha**, PH.D., University of California San Francisco

### Speaker Biosketch/Introduction

Dr. Ha is a postdoctoral fellow in the Delling laboratory at UCSF. She studies electric signaling in primary cilia using electrophysiology. My research is centered around the function of polycystin complex within primary cilia of renal epithelial cell. The title of her talk is "7 $\beta$ ,27-Dihydroxycholesterol activates the heteromeric PC-1/2 polycystin complex."

### Presentation Title

7 $\beta$ ,27-Dihydroxycholesterol activates the heteromeric PC-1/2 polycystin complex.

## Day 2 – Session 6

### Speaker/Institution

**Cristina Cebrian**, Ph.D., Assistant Professor, Division of Developmental Biology. Department of Pediatrics. Cincinnati Children's Hospital Medical Center. University of Cincinnati.

### Speaker/Institution

**Luis Menezes**, M.D., Ph.D., Staff Scientist, Polycystic Kidney Disease Section, Kidney Diseases Branch, NIDDK, NIH

## Polycystin Functions

### Speaker Biosketch/introduction

Dr. Cebrian is Assistant Professor in the Division of Developmental Biology, Department of Pediatrics at Cincinnati Children's Hospital Medical Center. The Cebrian lab has an established research program in kidney development and her current goal is to understand the earliest cellular and molecular mechanisms driving Autosomal Dominant Polycystic Kidney disease. The title of her talk is "Cyst clonality and identity in Autosomal Dominant Polycystic Kidney Disease"

### Speaker Biosketch/introduction

Dr. Menezes is a staff scientist in the polycystic kidney disease section in the Kidney diseases branch at NIDDK. His work applies omics approaches to animal models with a goal of understanding the initial steps of cystogenesis. The title of his talk is "Omics approaches to PKD".

### Presentation Title

Cyst clonality and identity in Autosomal Dominant Polycystic Kidney Disease

### Presentation Title

Omics Approaches to PKD

## Day 2 – Session 7

### Speaker/Institution

**Lloyd Cantley**, M.D.

### Speaker Biosketch/introduction

Dr. Cantley is the C. N. H. Long Professor of Medicine and Professor of Cellular and Molecular Physiology at the Yale School of Medicine. The primary focus of the Cantley lab is to determine the mechanisms of renal tubule development, homeostasis and repair. The title of his talk is "Physiologic and Pathologic Tubule remodeling"

### Presentation Title

Physiologic and Pathologic Tubule remodeling

**Speaker/Institution**

**Sorin Fedeles**, Ph.D., M.B.A.,  
Assistant Professor, Executive  
Director, Polycystic Kidney  
Disease Outcomes Consortium  
(PKDOC)  
Critical Path Institute, Rare and  
Orphan Diseases Program  
Assistant Professor (Adjunct)  
Yale School of Medicine

**Speaker Biosketch/introduction**

Sorin Fedeles is the Executive Director of the Polycystic Kidney Disease Outcomes Consortium (PKDOC) at the Critical Path Institute and an Assistant Professor (Adjunct) at Yale School of Medicine. His laboratory focuses on target identification and validation for polycystic kidney and liver diseases using whole-genome forward screens and pharmacological approaches. The title of his talk is “Modulation of hypomorphic polycystin-1 species *in vivo* ameliorates ADPKD progression”.

**Presentation Title**

Modulation of  
hypomorphic polycystin-1  
species *in vivo*  
ameliorates ADPKD  
progression