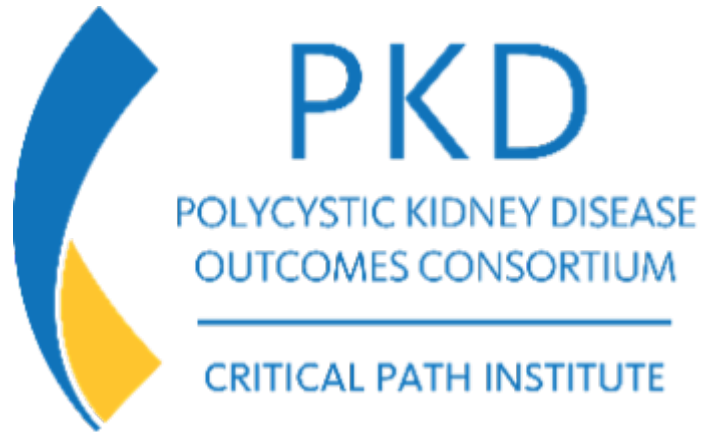


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PKD FOUNDATION
Polycystic Kidney Disease



The Critical Path Institute's Polycystic Kidney Disease Outcomes Consortium (PKDOC), The PKD Foundation and Otsuka invite you view the **2021 PKD Regulatory Summit**

The Summit included a series of three sessions with pre-recorded webinars available for registrants to view at their own pace two weeks prior to the meeting. On May 19 and 20, live presentations summarized content from the pre-recorded webinars, followed by panel discussions with speakers that facilitated dialogue among all participants.

[Access Live Presentations Day 1](#)

[Access Live Presentations Day 2](#)

Access pre-recorded presentations below.

AGENDA DAY 1: Wednesday, May 19

10 AM – 2 PM (ET)

SESSION 1: Advancing Drug Development Therapies for Autosomal Recessive Polycystic Kidney Disease (ARPKD)

SESSION 1: Pre-recorded Presentation Topics and Speakers		
1.	<u>Autosomal Recessive Polycystic Kidney Disease (ARPKD): Clinical course, trial endpoints, and imaging</u>	Erum Hartung, MD, MTR <i>Assistant Professor of Pediatrics, Division of Nephrology Children’s Hospital of Philadelphia (CHOP)</i>
2.	<u>ARegPKD Registry: Recent Developments</u>	Max Christoph Liebau, MD <i>Pediatric Nephrology & Center for Chronically Ill Children, Center for Molecular Medicine University of Cologne</i>
3.	<u>Translational Strategies for Autosomal Recessive Polycystic Kidney Disease (ARPKD)</u>	Lisa M. Guay-Woodford, MD <i>McGehee Joyce Professor of Pediatrics, Director, CTSI-CN/Director, Center for Translational Research Children’s National Research Institute</i>
4.	<u>Patient/Family Perspective</u>	Brandé and Ed Waldron, <i>ARPKD Parents</i>
5.	<u>Patient/Family Perspective</u>	Jaina and Lisa Cormack, <i>ARPKD Patient and Parent</i>
SESSION 1: Panelists		
<u>Moderators:</u>		
Ron Perrone, MD – <i>Tufts Medical Center</i>		
Lisa Guay-Woodford, MD – <i>Children’s National</i>		

Panelists:

Katherine Dell, MD – *Case Western Reserve University, Cleveland Clinic*

David Eiznhamer, PhD, MBA – *Kadmon Corporation*

Erum Hartung, MD, MTR – *Children’s Hospital of Philadelphia (CHOP)*

Max Liebau, MD – *University of Cologne*

Julie Marshall – *Patient speaker*

Kirtida Mistry, MD – *FDA*

Dimitar Roussinov, MD – *EMA, Pediatric Committee (PDCO)*

Joseph Toerner, MD, MPH – *FDA*

SESSION 2: Advancing Drug Development Therapies for Autosomal Dominant Polycystic Kidney Disease (ADPKD)

SESSION 2: Pre-recorded Presentation Topics and Speakers		
1.	<u>ADPKD: Diagnosis, Clinical Course, and health complications</u>	Neera Dahl, MD, PhD <i>Associate Professor, Section of Nephrology Yale University School of Medicine</i>
2.	<u>Advancing in the research of pediatric ADPKD</u>	Djalila Mekahli, MD, PhD <i>Pediatric Nephrology and Organ Transplantation University Hospital Leuven</i>
3.	<u>Assessing rapid progression in ADPKD</u>	Roser Torra, MD, PhD <i>Inherited Kidney Diseases, Nephrology Department Fundació Puigvert Barcelona, Spain</i>
4.	<u>Patient Perspective</u>	Lainie Esquivel, <i>ADPKD Patient</i>
5.	<u>Patient Perspective</u>	Karen Edwards, <i>ADPKD Patient</i>

SESSION 2: Panelists

Moderators:

Chris Rusconi, PhD – *PKD Foundation*

Arlene Chapman, MD – *University of Chicago*

Panelists:

Neera Dahl, MD, PhD – *Yale University School of Medicine*

Ali Hariri, MD – *Sanofi*

Pravin Jadhav, PhD – *Otsuka*

Romaldas Mažulaitis, MD, PharmD – *EMA*

Djalila Mekahli, MD, PhD – *University Hospital Leuven*

Kirtida Mistry, MD – *FDA*

Craig Ostroff, PharmD, R.Ph. – *Goldfinch Bio*

Norman Stockbridge, MD, PhD – *FDA*

Joseph Toerner, MD, MPH – *FDA*

Prof Roser Torra, MD, PhD – *Fundació Puigvert*

Hayley Womack – *Patient Speaker*

**2021 PKD Regulatory Summit
Agenda Day 1: Wednesday, May 19
10:00 AM – 2:00 PM (ET)**

10:00 – 10:10 am **DAY 1 INTRODUCTION: John-Michael Sauer**

SESSION 1: Advancing Drug Development Therapies for Autosomal Recessive Polycystic Kidney Disease (ARPKD)
Moderators: Ron Perrone and Lisa Guay-Woodford

10:10 – 10:20 am **Session 1 Introduction** Ron Perrone

10:20 – 10:35 am **Patient Speaker** “ARPKD Patient’s Perspective” Real life aspects of ARPKD
Julie Marshall

10:35 – 11:50 am **Session 1 Panel Discussion** *Featured speakers and panelists for discussion and Q&A about pre-recorded presentations*

11:50 am – 12:10 pm **BREAK (20 minutes)**

SESSION 2: Advancing Drug Development Therapies for Autosomal Dominant Polycystic Kidney Disease (ADPKD)
Moderators: Chris Rusconi and Arlene Chapman

12:10 – 12:20 pm **Session 2 Introduction** Chris Rusconi

12:20 – 12:35 pm **Patient Speaker** “ADPKD Patient’s Perspective” Real-life aspects of ADPKD
Hayley Womack

12:35 – 1:50 pm	Session 2 Panel Discussion	<i>Featured speakers and panelists for discussion and Q&A about pre-recorded presentations</i>
1:50 – 2:00 pm	DAY 1 WRAP-UP: John-Michael Sauer	

AGENDA DAY 2: Thursday, May 20 10 AM – 2:30 PM (ET)

SESSION 3: PKDOC 2.0: The Future of PKD Research

SESSION 3A: Data-driven Drug Development Tools

SESSION 3A: Pre-recorded Presentation Topics and Speakers		
1.	<p>Enabling a path to optimize clinical trial design in PKD:</p> <p>a) Clinical trial design in PKD: Early TKV modeling for trial enrichment</p> <p>b) From biomarkers, to surrogates, to optimized PKD trials</p>	<p>Vicente E. Torres, MD, PhD <i>Director, Translational Polycystic Kidney Disease Center Mayo Clinic</i></p> <p>Klaus Romero, MD, MS, FCP <i>Chief Science Officer Critical Path Institute</i></p>
2.	Clinical Outcome Assessments in ADPKD	<p>Dorothee Oberdhan, MS <i>Director, Patient-Centric Outcomes Global Value & Real-World Evidence Otsuka Pharmaceuticals</i></p>
3.	Potential Role of C-Path’s Clinical Outcome Assessment (COA) Program	<p>Rebecca Speck, PhD, MPH <i>Clinical Outcome Assessment (COA) Scientist, COA Program Critical Path Institute</i></p>
4.	Data Sharing – Value Proposition for the future of Rare Disease R&D	<p>Jeff Barrett, PhD, FCP <i>Senior Vice President, RDCA-DAP Lead Critical Path Institute</i></p>
5.	Lessons Learned Over Nearly Two Decades of CKD-EPI	<p>Lesley A. Inker, MD, MS, FRCP <i>Associate Professor Tufts Medical Center Tufts University School of Medicine</i></p>
6.	Patient Perspective	<p>Heidi Cambareri, <i>ADPKD Patient</i></p>

SESSION 3A: Panelists

Moderators:

John-Michael Sauer, PhD – *Critical Path Institute*

Frank Czerwiec, MD, PhD – *Goldfinch Bio*

Panelists:

Jeff Barrett, PhD – *Critical Path Institute*

Ali Hariri, MD – *Sanofi*

Lesley Inker, MD, MS, FRCP – *Tufts University School of Medicine*

Pravin Jadhav, PhD – *Otsuka*

Romaldas Ma?iulaitis, MD, PharmD – *EMA*

Cari Maxwell – *Patient Speaker*

Dorothee Oberdhan, MS – *Otsuka*

Klaus Romero, MD, MS, FCP – *Critical Path Institute*

Rebecca Speck, PhD, MPH – *Critical Path Institute*

Aliza Thompson, MD – *FDA*

Vicente Torres, MD, PhD – *Mayo Clinic*

SESSION 3B: Toward a Regulatory Standard for ADPKD

SESSION 3B: Pre-recorded Presentation Topics and Speakers

1.	Study proposal for a PKD clinical trial	Frank S. Czerwiec, MD, PhD <i>Chief Medical Officer</i> Goldfinch Bio Ronald D. Perrone, MD <i>Division of Nephrology</i> <i>Scientific Director, Clinical and</i> <i>Translational Research Center</i> Tufts Medical Center Tufts University School of Medicine
2.	Patient Perspective	Greg Mainolfi, <i>ADPKD Patient</i>

SESSION 3B: Panelists

Moderators:

Frank Czerwiec, MD, PhD – *Goldfinch Bio*

Aliza Thompson, MD – *FDA*

Romaldas Mažiulaitis, MD, PharmD – *EMA*

Panelists:

Karl Cremer, PharmD – *Regulus Therapeutics*

Peter Cody Fiduccia – *Patient Speaker*

Kevin Fowler – *The Voice of the Patient Inc.*

Ken Gruchalla, MD – *Health Canada*

Tess Harris – *PKD Charity*

Neil Shusterman, MD – *Palladio Biosciences*

Liron Walsh, MD – *Goldfinch Bio*

Alan Yu, M.B. B.Chir. – *University of Kansas School of Medicine*

2021 PKD Regulatory Summit Agenda Day 2: Thursday, May 20 10:00 AM – 2:30 PM (ET)

10:00 – 10:10 am	DAY 2 INTRODUCTION: John-Michael Sauer	
SESSION 3A: PKDOC 2.0: The Future of PKD Research – Data-driven Drug Development Tools Moderators: John-Michael Sauer and Frank Czerwiec		
10:10 – 10:15 am	Session 3A Introduction	John-Michael Sauer
10:15 – 10:30 am	Patient Speaker	Cari Maxwell
10:30 – 11:40 am	Session 3A Panel Discussion	<i>Featured speakers and panelists for discussion and Q&A about pre-recorded presentations</i>
11:40 am – 12:00 pm	BREAK (20 minutes)	
SESSION 3B: PKDOC 2.0: The Future of PKD Research – Toward a Regulatory Standard for ADPKD Moderators: Frank Czerwiec, Aliza Thompson and Romaldas Mažiulaitis		
12:00 – 12:05 pm	Session 3B Introduction	Frank Czerwiec

12:05 – 12:20 pm	Patient Speaker	Peter Cody Fiduccia
12:20 – 12:35 pm	Overview of protocol	Frank Czerwiec
12:35 – 1:05 pm	Regulatory Comment	Aliza Thompson and Romaldas Mažiulaitis
1:05 – 2:15 pm	Session 3B Panel Discussion	<i>Featured speakers and panelists for discussion and Q&A about pre-recorded presentations</i>
2:15 – 2:30 pm	<u>CLOSING COMMENTS</u>: John-Michael Sauer	

Funding for the Summit provided by The PKD Foundation and Otsuka.