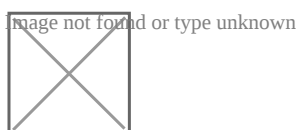
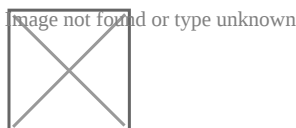


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The Critical Path Institute's Polycystic Kidney Disease Outcomes Consortium (PKDOC), The PKD Foundation and Otsuka invite you view the **2021 PKD Regulatory Summit**

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.	Autosomal Recessive Polycystic Kidney Disease (ARPKD): Clinical course, trial endpoints, and imaging	Erum Hartung, MD, MTR <i>Assistant Professor of Pediatrics, Division of Nephrology Children's Hospital of Philadelphia (CHOP)</i>

2.	ARegPKD Registry: Recent Developments	Max Christoph Liebau, MD <i>Pediatric Nephrology & Center for Chronically Ill Children, Center for Molecular Medicine</i> University of Cologne
3.	Translational Strategies for Autosomal Recessive Polycystic Kidney Disease (ARPKD)	Lisa M. Guay-Woodford, MD <i>McGehee Joyce Professor of Pediatrics, Director, CTSI-CN/Director, Center for Translational Research</i> Children's National Research Institute
4.	Patient/Family Perspective	Brandé and Ed Waldron, <i>ARPKD Parents</i>
5.	Patient/Family Perspective	Jaina and Lisa Cormack, <i>ARPKD Patient and Parent</i>

SESSION 1: Panelists

Moderators:

Ron Perrone, MD – *Tufts Medical Center*

Lisa Guay-Woodford, MD – *Children's National*

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.	ADPKD: Diagnosis, Clinical Course, and health complications	Neera Dahl, MD, PhD <i>Associate Professor, Section of Nephrology</i> Yale University School of Medicine
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2.	Advancing in the research of pediatric ADPKD	Djalila Mekahli, MD, PhD <i>Pediatric Nephrology and Organ Transplantation</i> University Hospital Leuven
3.	Assessing rapid progression in ADPKD	Roser Torra, MD, PhD <i>Inherited Kidney Diseases, Nephrology Department</i> Fundació Puigvert Barcelona, Spain
4.	Patient Perspective	Lainie Esquivel, <i>ADPKD Patient</i>
5.	Patient Perspective	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 Ma?iulaitis, 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	Featured speakers and panelists for discussion and Q&A about pre-recorded presentations
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.	Enabling a path to optimize clinical trial design in PKD: a) Clinical trial design in PKD: Early TKV modeling for trial enrichment b) From biomarkers, to surrogates, to optimized PKD trials	Vicente E. Torres, MD, PhD Director, Translational Polycystic Kidney Disease Center Mayo Clinic Klaus Romero, MD, MS, FCP Chief Science Officer Critical Path Institute
2.	Clinical Outcome Assessments in ADPKD	Dorothee Oberdhan, MS Director, Patient-Centric Outcomes Global Value & Real-World Evidence Otsuka Pharmaceuticals

3.	Potential Role of C-Path's Clinical Outcome Assessment (COA) Program	Rebecca Speck, PhD, MPH <i>Clinical Outcome Assessment (COA) Scientist, COA Program</i> Critical Path Institute
4.	Data Sharing – Value Proposition for the future of Rare Disease R&D	Jeff Barrett, PhD, FCP <i>Senior Vice President, RDCA-DAP Lead</i> Critical Path Institute
5.	Lessons Learned Over Nearly Two Decades of CKD-EPI	Lesley A. Inker, MD, MS, FRCP <i>Associate Professor</i> Tufts Medical Center Tufts University School of Medicine
6.	Patient Perspective	Heidi Cambareri, <i>ADPKD Patient</i>

SESSION 3A: Panelists

Moderators:

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

Frank Czerwicz, 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 Translational Research Center</i> Tufts Medical Center Tufts University School of Medicine
2.	Patient Perspective	Greg Mainolfi, ADPKD Patient

SESSION 3B: Panelists	
<u>Moderators:</u> Frank Czerwiec, MD, PhD – <i>Goldfinch Bio</i> Aliza Thompson, MD – <i>FDA</i> Romaldas Mačiulaitis, MD, PharmD – <i>EMA</i>	
<u>Panelists:</u> Karl Cremer, PharmD – <i>Regulus Therapeutics</i> Peter Cody Fiduccia – <i>Patient Speaker</i> Kevin Fowler – <i>The Voice of the Patient Inc.</i> Ken Gruchalla, MD – <i>Health Canada</i> Tess Harris – <i>PKD Charity</i> Neil Shusterman, MD – <i>Palladio Biosciences</i> Liron Walsh, MD – <i>Goldfinch Bio</i> Alan Yu, M.B. B.Chir. – <i>University of Kansas School of Medicine</i>	

2021 PKD Regulatory Summit Agenda Day 2: Thursday, May 20 10:00 AM – 2:30 PM (ET)		
10:00 – 10:10 am	<u>DAY 2 INTRODUCTION: John-Michael Sauer</u>	
<u>SESSION 3A: PKDOC 2.0: The Future of PKD Research – Data-driven Drug Development Tools</u> 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	Featured speakers and panelists for discussion and Q&A about pre-recorded presentations
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 Mačiulaitis		
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 Mačiulaitis
1:05 – 2:15 pm	Session 3B Panel Discussion	Featured speakers and panelists for discussion and Q&A about pre-recorded presentations
2:15 – 2:30 pm	CLOSING COMMENTS: John-Michael Sauer	

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