View Now | 2021 PKD Regulatory Summit | May 19-20

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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 *Assistant Professor of Pediatrics, Division of Nephrology* Children's Hospital of Philadelphia (CHOP)

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

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 TopicsTopics and Speakers		
1.	ADPKD: Diagnosis, Clinical Course, and health complications	Neera Dahl, MD, PhD *Associate Professor, Section of Nephrology* Yale University School of Medicine

2.	Advancing in the research of pediatric ADPKD	Djalila Mekahli, MD, PhD *Pediatric Nephrology and Organ Transplantation* University Hospital Leuven
3.	Assessing rapid progression in ADPKD	Roser Torra, MD, PhD *Inherited Kidney Diseases, Nephrology Department* Fundació Puigvert Barcelona, Spain
4.	Patient Perspective	Lainie Esquivel, *ADPKD Patient*
5.	Patient Perspective	Karen Edwards, *ADPKD Patient*

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

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 *Chief Medical Officer* Goldfinch Bio Ronald D. Perrone, MD *Division of Nephrology* *Scientific Director, Clinical and Translational Research Center* Tufts Medical Center Tufts University School of Medicine
2.	Patient Perspective	Greg Mainolfi, *ADPKD Patient*

SESSION 3B: Panelists
Moderators: Frank Czerwiec, MD, PhD – *Goldfinch Bio* Aliza Thompson, MD – *FDA* Romaldas Ma?iulaitis, 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**	*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 Ma?iulaitis		
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 Ma?iulaitis
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.