VIRTUAL SCIENTIFIC CONFERENCE
2022 PROGRAM

PCD ON THE MOVE

AUGUST 4 & 9, 2022

PCD FOUNDATION

WELCOME

Welcome to the 2022 'PCD on the Move'. We are delighted you can join us virtually for an update on the remarkable progress being made in PCD research. While we wish we were together in person, we anticipate this will remain an interactive, dynamic meeting and look forward to your questions, comments and feedback.

The pace of research in PCD has accelerated exponentially in the past decade and we are excited at the prospect of translating what we've learned into effective treatments and ultimately cure(s) for PCD. This is reflected in the program for 2022, which features robust presentations highlighting new discoveries. Additionally, the latest information on improving PCD diagnosis and treatment will be presented, including updates from international colleagues and industry collaborators. We are grateful for the support of our speakers, moderators and presenters.

As always, this meeting would not be possible without the support of our generous sponsors:

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On behalf of the PCDF Board of Directors, I extend a warm welcome to this year's 'PCD on the Move'.

Michele Manion
President & Executive Director, PCD Foundation

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Conference Objectives:

1. Enable attendees to optimize clinical care of patients with PCD by providing education, access to experts and hands-on learning opportunities for recent advances in PCD to include:
   a. Evolving gene identification
   b. Emerging phenotypic features/overlapping syndromes
   c. Novel therapeutic options
2. Encourage expansion of existing clinical care and research networks with the goal of accelerating clinical research trials
3. Identify clinical research opportunities and prioritize research goals

Confidentiality:

This PCDF On the Move meeting will include presentations and discussions that are confidential. Please do not share information from oral/written presentations, posters, abstracts, or recorded discussions without the express permission of the presenter.

PROGRAM AGENDA: DAY 1

PCD on the Move 2022!

Thursday, August 4th 8:45 AM – 1:40 PM (All times US Eastern Daylight Savings Time)

Welcome: Day 1		
8:45-8:50	PCD Foundation	Michele Manion, Executive Director, PCD Foundation
8:50-9:00	Conference Chairs	Margaret Leigh, MD; Deepika Polineni, MD
Moving Towards PCD Therapies		
9:00-9:25	Clinical Studies & Clinical Trials Progress to Date	Stephanie Davis, MD, University of North Carolina
9:25-9:50	Clinical Studies & Clinical Trials Overview: Trials in the Pipeline Panel Discussion	Margaret Leigh, University of North Carolina
9:50-10:15	Developing a Clinical Trials Management Network	Kim Nielsen, MD, University of Copenhagen
10:15-10:30	Discussion	Group
Break (15 Minutes)		
10:45-11:30	Keynote: Phenotyping and Endotyping in non-CF Bronchiectasis	James Chalmers, MD, University of Dundee

PCD Scientific Reports		
Session Chairs: Evans Machogu, MD: Amjad Horani, MD		
11:30-11:35	Introduction	Session Chairs
11:35-11:50	Therapeutic Oligonucleotides to Correct Splicing Mutations Causing Primary Ciliary Dyskinesia	Silvia Kreda, PhD, University of North Carolina
11:50-12:05	Genotype to Phenotype: Measuring Mucociliary Clearance in Primary Ciliary Dyskinesia	Sara Abu-Nasser, DO, University of North Carolina
12:05-12:20	Genetics and Olfactory Dysfunction in Primary Ciliary Dyskinesia	Zainab Farzal, MD, University of North Carolina
12:20-12:35	Lung Function in Primary Ciliary Dyskinesia by Genotype: DNAH11 versus other outer dynein arm genes	Kunal Jakharia, MD, University of North Carolina
12:35-12:50	Heterotaxy Syndrome and Primary Ciliary Dyskinesia: Outcomes for Infants with Complex Congenital Heart Disease	Jonathan Marquez, MD, PhD, University of Washington
12:50-1:05	Microbiological Profiling and BAL Immunophenotype of PCD Patients with the Puerto Rican RSPH4A Founder Mutation	Andre Marra Nazario, BS, Ponce Health Sciences University
1:05-1:120	HYDIN in plain sight: A Seemingly Rare Primary Ciliary Dyskinesia Gene is Common in French-Canadians	Adam Shapiro, MD, McGill University
1:20-1:35	Intra-day reliability of multiple breath washout in pediatric primary ciliary dyskinesia	Wallace Wee, MD, University of Toronto
Closing Remarks: Michele Manion, Executive Director, PCDF		

PROGRAM AGENDA: DAY 2

PCD on the Move 2022!

Tuesday, August 9th, 11:45 AM –4:30 PM (All times US Eastern Daylight Savings Time)

Welcome: Day 2		
11:45-11:50	PCD Foundation	Michele Manion, Executive Director, PCD Foundation
11:50-12:00	Conference Chairs	Margaret Leigh, MD; Deepika Polineni, MD
Moving towards Genetic Therapies		
12:00-12:25	Challenges of Genetic Diagnosis	Marie Legendre, PhD, PharmD, Sorbonne University
12:25-12:50	PCD Diagnosis: The Puerto Rican Experience	Wilfredo De Jesus Rojas, MD, FAAP, MSc Ponce Health Science University
12:50-1:15	Primary Ciliary Dyskinesia Among Children with Heterotaxy	Tom Saba, MD, Mott Children's Hospital
1:15-1:40	Strategies for Personalized Treatment of PCD	Larry Ostrowski, PhD, University of North Carolina
Break (10 Minutes)		
1:50-2:35	Keynote: Regenerative medicine approaches to model and treat PCD	Finn Hawkins, MBBch, Boston University School of Medicine
2:35-2:50	Trial Strategies for Small Populations: CF Modulator Experience	Deepika Polineni, MD, Washington University
Break (10 Minutes)		
3:00-3:25	PCDF Registry Updates	Michael O'Connor, MD, Vanderbilt University

CRCN and Extended Network Case Reports		
Session Chairs: Hili Metjen, MD; Catherine Sanders, MD		
3:25-3:30	Introduction	Session Chairs
3:30-3:45	Twins with pathogenic variants in CCNO causing PCD: Interplay of Pulmonary and Gastrointestinal Manifestations	Alex Despostes, MD, University of North Carolina
3:45-4:00	Persistence pays off: expanding genetic analyses to identify disease-causing variants in previously discovered genes in primary ciliary dyskinesia (PCD).	R. Drew Gardner, MD, University of North Carolina
4:00-4:15	Solving the PCD Puzzle: Are ABC9A or FAM105A Genes New Missing Pieces?	Jesus Melendez-Montañez, BS, Ponce Health Sciences University
4:15-4:30	A deep intronic, pathogenic variant in DNAH11 causes Primary Ciliary Dyskinesia	Maimoona Zariwala, PhD, University of North Carolina
Closing Remarks: Michele Manion, Executive Director, PCDF		

SPEAKER LIST

Sara Abu-Nasser, DO
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Margaret Leigh, MD
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Andre Marra Nazario, BS
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Jonathan Marquez, MD, PhD
Pediatrics – Medical Genetics & Genomics Resident
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SPEAKER LIST

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Deepika Polineni, MD
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2022 VIRTUAL EXHIBITORS

Parion Sciences
www.parion.com

Parion Sciences is a development stage biopharmaceutical company dedicated to research, development, and commercialization of treatments to improve and extend the lives of patients with severe respiratory diseases. Parion has a diverse pipeline of pre-clinical and clinical candidates for the treatment of these diseases via distinctive mechanisms of action and approaches. Parion's lead program, Idrevloride (formerly P-1037) Inhalation Solution, is a novel, inhaled, long acting ENaC Inhibitor. Idrevloride Inhalation Solution has been shown to improve airway mucus hydration and allow for more efficient mucus clearance in patients with PCD, thus leading to improved lung function. Idrevloride Inhalation Solution is the first treatment in development to exclusively treat PCD patients and preparations are ongoing to initiate Phase 3 development. Parion has received support and grant funding from the National Institutes of Health and the Cystic Fibrosis Foundation.

For more information:
Paul Boucher, President & CEO
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2022 VIRTUAL EXHIBITORS

Rho
https://www.rhoworld.com/

Rho is a global, privately held contract research organization (CRO) headquartered in Research Triangle Park. Rho provides a full range of drug development services, from program strategy through to clinical trials and marketing applications. For more than 36 years, Rho has been a trusted partner to some of the most innovative pharmaceutical, biotechnology and medical device companies as well as academic and government organizations. With over 30,000 rare disease participants treated in more than 150 studies and 2,400 sites, along with our own personal experiences supporting friends and family struggling with rare diseases, Rho not only feels their pain – we're determined to help find the cure. Experience Rho by following the company on Facebook, LinkedIn, Twitter, and Instagram.

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Running On Air
https://runningonair.org

Running On Air (ROA) is a 501(c)(3) charitable organization of volunteers whose mission is to help others breathe more easily. ROA's focus is on advocacy and education around supplemental oxygen, lung diseases, especially Primary Ciliary Dyskinesia (PCD) and Bronchiectasis, and rare diseases. Learn more by following ROA on Facebook, Twitter and Instagram.

Registration is now open for ROA's Move 4 A Cure virtual race celebrating the PCD Foundation's 20th anniversary. This event takes place throughout the month of October, which is PCD Awareness Month. Registration information available here.

For more information:
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Stay tuned for information about
PCD On the Move 2023!

PCD FOUNDATION™

PRIMARY CILIARY DYSKINESIA

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