



BEAT-PCD
BETTER EXPERIMENTAL APPROACHES TO TREAT PCD



European
Reference
Network

ERN-LUNG
European Reference Network
for rare respiratory diseases

PCD Annual Research Meeting 2023

September 8th, 2023

**University of Milano, Via Festa del Perdono 3
Meeting Room n. 302**

Organising Secretariat



AUDIENCE

Researchers, Clinicians, Scientists, AHPs and early career members with an interest in diagnosis and management of the inherited motile ciliopathy Primary ciliary dyskinesia

BEAT-PCD

is a European Respiratory Society clinical research collaboration. The long term vision of BEAT-PCD is that children with PCD born anywhere will receive an early accurate diagnosis and evidence based PCD specific therapies which will prevent development of bronchiectasis and will reduce morbidity and mortality.

ERN-LUNG

The European Reference Network for rare respiratory diseases, is a network of European healthcare providers (HCP) dedicated to ensuring and promoting excellence in care and research for the benefit of patients affected by rare respiratory diseases. The ERNs bring together specialised research centres to combine expertise in the field and aim to provide all patients with rare diseases better access to quality diagnosis, treatment and care, even beyond borders.

Meeting Objectives:

- Update members of the PCD community on the latest research and European initiatives
- Provide opportunity to get involved in shared projects and goals and build on important international collaborations, crucial in rare disease research
- Involve and encourage early career members in PCD research
- Learn from people with PCD about research priorities: patients are central to our objectives

PCD Annual Research Meeting 2022 Group



Organising Secretariat

MORNING TRAINING PROGRAM

- 09.00 **Welcome and introduction** - Chairs: Myrona Goutaki, Heymut Omran Maria Francesca Patria (Italian Representative), Amelia Shoemark, Myrona Goutaki (BEAT-PCD representatives), Johanna Raidt, Heymut Omran (ERN-LUNG representatives)
- 09.10 **Patient perspective: Italian patient representative** - (Associazione italiana discinesia ciliare primaria - sindrome die Kartagener Aps)
- 09.20 **Patient perspective: BEAT-PCD patient representative** - Lucy Dixon (PCD Support UK)
- 09.30 **Italian Perspective** - Nicola Ullmann
- 09.45 **Training talks/interactive session** - Chairs: Rob Hirst, Nicola Ullman
- Assessment of human airway ciliary beating for the diagnosis of Primary Ciliary Dyskinesia- Mathieu Bottier (20min including questions)
 - Panel discussion: What's needed for multi-disciplinary management in PCD? Open discussion with a multidisciplinary panel of patient representatives and healthcare professionals involved in the care of people with PCD (25min discussion)
- 10.30 **Research highlights from selected early career member abstracts** - Chairs: Katie Horton, Yin Ting Lam
This session will provide a platform for people with ERS posters to give short oral presentations of their work (5min each followed by 15min joint discussion)
- Variability of lung function in patients with primary ciliary dyskinesia (PCD): longitudinal analysis from the PROVALF-PCD cohort- Bruna Rubbo
 - Assessing personalised airway clearance techniques in Primary Ciliary Dyskinesia with 129Xe ventilation MRIXX- Lynne Schofield
 - Evaluation of sleep disordered breathing and related factors in primary ciliary dyskinesia patients- Nagehan Emiralioglu
 - Infertility among adults with primary ciliary dyskinesia- Leonie Schreck
 - The importance of collaboration with expert centers on PCD diagnosis in limited-resource countries- Mine Kalyoncu
- 11.10 **Coffee break**
- 11.30 **Difficult diagnosis and management cases presentations** - Chairs: Amjad Horani, Panayiotis Kouis
Difficult cases (4 cases x10min + 5min discussion each)
- CFAP74 mutations or NEK10 mutations- Luisa Biebach
 - Combined approaches including long-read sequencing address the diagnostic challenge of HYDIN in primary ciliary dyskinesia - Debbie Morris-Rosendahl
 - More tbd – You are invited to contact us if you would like to present a difficult case.

12.30 **Lunch**

AFTERNOON EUROPEAN PCD MEETING

- 13.30 **Plenary Talk** - Chairs: Claudia Kuehni, Kim Nielsen
The challenges of monitoring disease in people with PCD - Jane Lucas (30min including discussion)
- 14.00 **Nasal nitric oxide measurement in children for the diagnosis of PCD: ERS technical standard**-Nicole Beydon (15mins + 5min discussion)
- 14.20 **Clinical trials updates** - Chairs: Felix Ringshausen, Phil Robinson (15mins + 5min discussion each)
- ReCode Therapeutics Update- John Matthews
 - Ethris GmbH Update- Thomas Langenickel
 - Mucociliary Clearance Assays - Pulmonary Radioaerosol Mucociliary Clearance - June K. Marthin
 - CLEAN-PCD trial results- Felix Ringshausen
- 15.40 **Coffee break**
- 16.10 **Update on European and international PCD projects** - Chairs: Amelia Shoemark, Petra Pennekamp (15min each including questions)
- Update on the US PCD network - Tom Ferkol
 - Update on the ERN-LUNG PCD Clinical Trials Network - Kim Nielsen
 - Update on the ERN-LUNG PCD international PCD registry and audits - Johanna Raidt
 - Update on BEAT-PCD - Myrona Goutaki
 - Plans for next 3 years of BEAT-PCD - Amelia Shoemark

17.30 **Meeting close and summary**

18.00 **Get together with some snacks and drinks**

Organising Secretariat

For more information on BEAT-PCD
beat-pcd.squarespace.com

For more information on ERN-LUNG
ern-lung.eu

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