

association



dcp

dyskinésie ciliaire primitive

Conseil scientifique

09/06/2023

Actualités Recherche

B Maitre

- Génétique qs exposé R Mani/M Legendre
- **Etudes publiées**
- Etudes en cours
- Renouvellement CRC BEAT PCD
- Clinical Trial Network

Etudes publiées

- Recommandations pour les techniques diagnostiques
 - Mesure du NO nasal chez l'enfant



EUROPEAN RESPIRATORY JOURNAL
ERS OFFICIAL DOCUMENTS
N. BEYDON ET AL.

Nasal nitric oxide measurement in children for the diagnosis of primary ciliary dyskinesia: European Respiratory Society technical standard

Nicole Beydon ^{1,2,24}, Panayiotis Kouis ³, June K. Marthin ⁴, Philipp Latzin ⁵, Murielle Colas⁶,
Stephanie D. Davis⁷, Eric Haarman⁸, Amanda Lea Harris ^{9,10}, Claire Hogg¹¹, Emma Kilbride¹²,
Claudia E. Kuehni ^{5,13}, Diana Marangu¹⁴, Kim G. Nielsen^{4,15}, Catherine Pendergrast ^{16,17},
Phil Robinson ^{18,19,20}, Nisreen Rumman^{21,22}, Matthew Rutter²³, Woolf T. Walker^{9,10}, Thomas Ferkol⁷ and
Jane S. Lucas ^{9,10,24}

Index de clairance pulmonaire

[Chest Infections Original Research]










Comparison of the Lung Clearance Index in Preschool Children With Primary Ciliary Dyskinesia and Cystic Fibrosis



*Jobst F. Roehmel, MD; Friederike J. Doerfler; Cordula Koerner-Rettberg, MD; Folke Brinkmann, MD;
Anne Schlegtendal, MD; Martin Wetzke, MD; Isa Rudolf, MD; Simone Helms; Joerg Große-Onnebrink, MD;
Yin Yu, MD; Thomas Nuesslein, MD; Irena Wojsyk-Banaszak, MD; Sebastian Becker, MD; Olaf Eickmeier, MD;
Olaf Sommerburg, MD; Heymut Omran, MD; Mirjam Stahl, MD; and Marcus A. Mall, MD*

COVID-PCD

COVID-PCD: a participatory research study on the impact of COVID-19 in people with primary ciliary dyskinesia

Eva S.L. Pedersen ¹, Eugénie N.R. Collaud¹, Rebeca Mozun ^{1,2},
Cristina Ardura-Garcia ¹, Yin Ting Lam^{1,2}, Amanda Harris ³, Jane S. Lucas ^{3,4},
Fiona Copeland⁵, Michele Manion⁶, Bernhard Rindlisbacher⁷,
Hansruedi Silberschmidt⁸, COVID-PCD patient advisory group,
Myrofora Goutaki ^{1,9} and Claudia E. Kuehni ^{1,9}

Atteinte ORL

Sinonasal disease among patients with primary ciliary dyskinesia: an international study

Yin Ting Lam ^{1,2}, Jean-François Papon^{3,4}, Mihaela Alexandru ^{3,4}, Andreas Anagnostos⁵, Miguel Armengot^{6,7}, Mieke Boon⁸, Andrea Burgess⁹, Suzanne Crowley ¹⁰, Sinan Ahmed D. Dheyauldeen^{11,12}, Nagehan Emiralioglu ¹³, Ela Erdem Eralp¹⁴, Christine van Gogh¹⁵, Yasemin Gokdemir¹⁴, Onder Gunaydin¹⁶, Eric G. Haarman¹⁷, Amanda Harris ^{18,19}, Isolde Hayn²⁰, Hasnaa Ismail-Koch⁹, Bülent Karadag ¹⁴, Céline Kempeneers ²¹, Sookyung Kim³, Philipp Latzin ²², Natalie Lorent ²³, Ugur Ozcelik¹³, Charlotte Pioch²⁴, Anne-Lise M.L. Poirrier ²⁵, Ana Reula^{26,27}, Jobst Roehmel ²⁴, Panayiotis Yiallourous^{28,29}, on behalf of the EPIC-PCD team³⁰, and Myrofora Goutaki ^{1,22}

Atteinte ORL

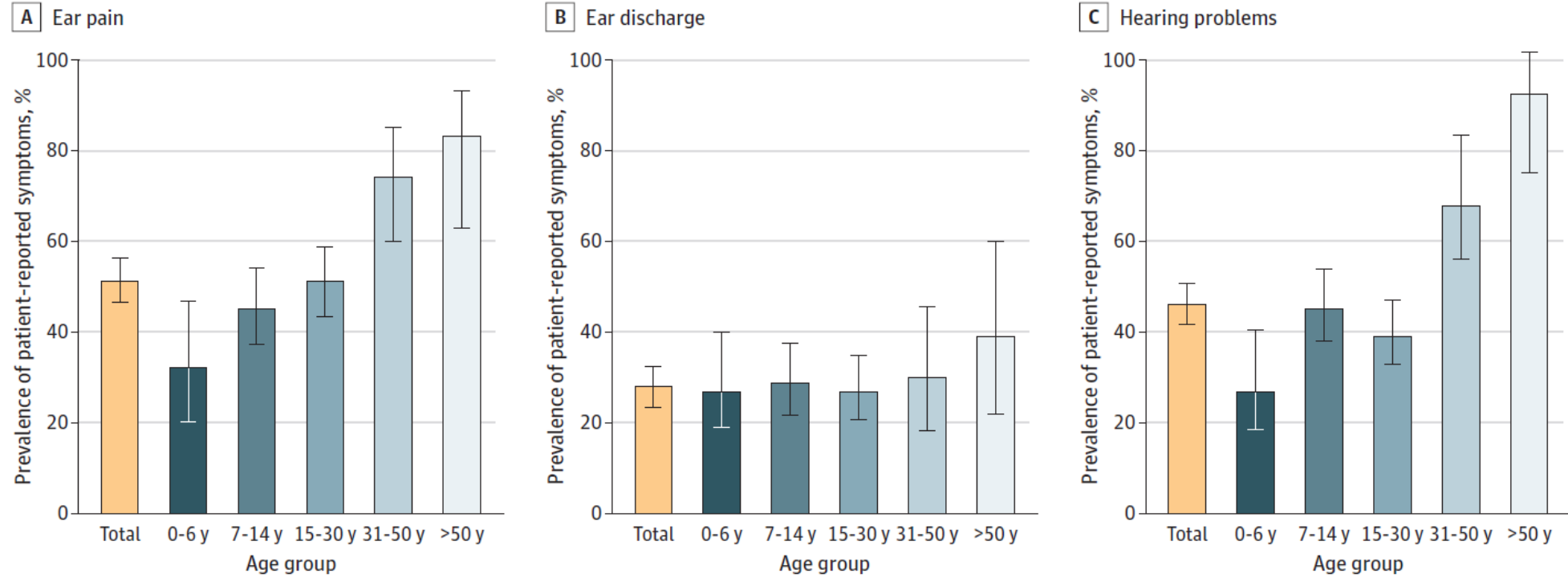
Research

JAMA Otolaryngology–Head & Neck Surgery | [Original Investigation](#)

Characteristics of Otologic Disease Among Patients With Primary Ciliary Dyskinesia

Myrofora Goutaki, MD, PhD; Yin Ting Lam, MD; Mihaela Alexandru, MD, MSc; Andreas Anagnostos, MD, PhD; Miguel Armengot, MD, PhD; Mieke Boon, MD, PhD; Andrea Burgess, MD; Nathalie Caversaccio, MD; Suzanne Crowley, MD; Sinan Ahmed D. Dheyauldeen, MD, PhD; Nagehan Emiralioglu, MD; Ela Erdem, MD; Christine van Gogh, MD; Onder Gunaydin, MD; Eric G. Haarman, MD, PhD; Amanda Harris, MSc; Isolde Hayn, MD; Hasnaa Ismail-Koch, MD; Bulent Karadag, MD; Céline Kempeneers, MD, PhD; Sookyung Kim, MD; Natalie Lorent, MD; Ugur Ozcelik, MD; Charlotte Pioch; Anne-Lise M. L. Poirrier, MD, PhD; Ana Reula, PhD; Jobst Roehmel, MD; Panayiotis Yiallouros, MD, PhD; Ali Cemal Yumusakhuylu, MD; Jean-François Papon, MD, PhD

Figure 1. Prevalence of Self- and Parent-Reported Ear Symptoms Among EPIC-PCD Participants, Overall and by Age Group (N = 397)



EPIC-PCD indicates the Ear-Nose-Throat Prospective International Cohort of patients with primary ciliary dyskinesia. Error bars represent 95% CIs.

Table 3. Audiometric Findings Among EPIC-PCD Participants, Overall and by Age Group

Hearing loss grade ^a	No. (%)						Affected ears (N = 546)
	Total (N = 273)	Age 0-6 y (n = 18)	Age 7-14 y (n = 84)	Age 15-30 y (n = 110)	Age 31-50 y (n = 41)	Age >50 y (n = 20)	
Normal hearing (≤ 25 dB)							
Bilateral	154 (56)	7 (39)	53 (63)	77 (70)	17 (41)	0	341 (63)
Unilateral	33 (12)	1 (6)	12 (14)	14 (13)	4 (10)	1 (5)	
Mild hearing loss (26-40 dB)							
Bilateral	55 (20)	8 (44)	12 (14)	13 (12)	14 (34)	8 (40)	158 (29)
Unilateral	48 (18)	1 (6)	15 (18)	17 (15)	9 (22)	6 (30)	
Moderate hearing loss (41-60 dB)							
Bilateral	7 (3)	1 (6)	2 (2)	0	1 (2)	3 (15)	35 (6)
Unilateral	21 (8)	1 (6)	4 (5)	6 (5)	5 (12)	5 (25)	
Severe hearing loss (61-80 dB)							
Bilateral	1 (0)	0	0	0	0	1 (5)	6 (1)
Unilateral	4 (1)	0	1 (1)	0	0	3 (15)	
Profound hearing loss (≥ 81 dB)							
Bilateral	1 (0)	0	1 (1)	0	0	0	3 (0)
Unilateral	1 (0)	0	0	1 (1)	0	0	
Could not be performed ^b	3 (1)	NA	NA	NA	NA	NA	3 (0)

Abbreviations: EPIC-PCD, Ear-Nose-Throat Prospective International Cohort of patients with primary ciliary dyskinesia; NA, not applicable.

^a Hearing loss grades are based on the World Health Organization hearing loss grading system. Categories are not exclusive because participants can have

different hearing loss grades in each ear.

^b In 3 patients, audiometry was recorded as not performed due to technical reasons for 1 of the ears.

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COVID PCD Fertility

- En cours de publication
 - 300-400 répondants
 - Information et accès centres de fertilité
 - Données efficacité, grossesse ectopique

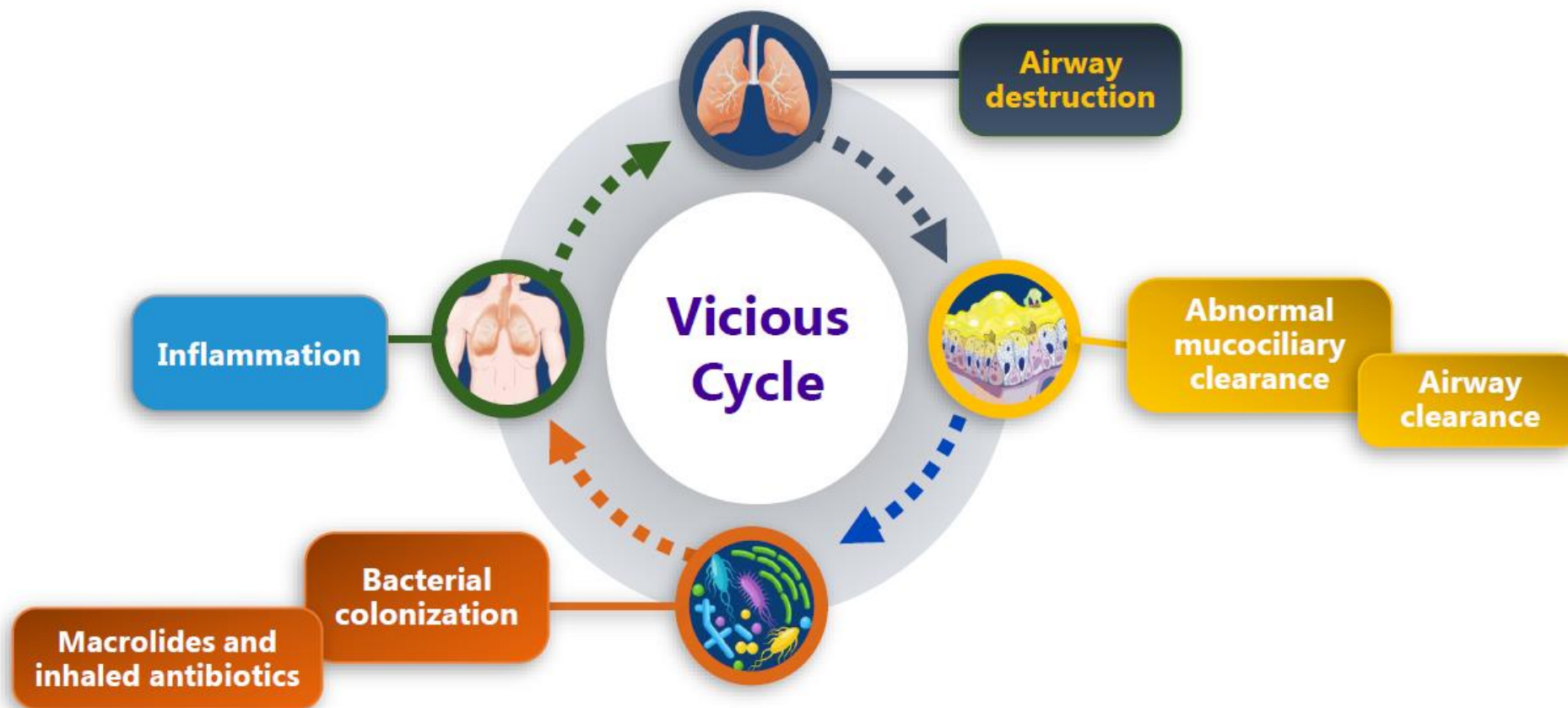
Autres études en cours

- Définition exacerbations ORL Renata Kos
- Diagnostic de DCP dans une cohorte au Royaume Uni, screening par génétique directement Amelia Shoemark
 - Sous évaluation du diagnostic
- Etude sur les patients de la même fratrie (corrélation phenotype/genotype) Panayotis Yallouros

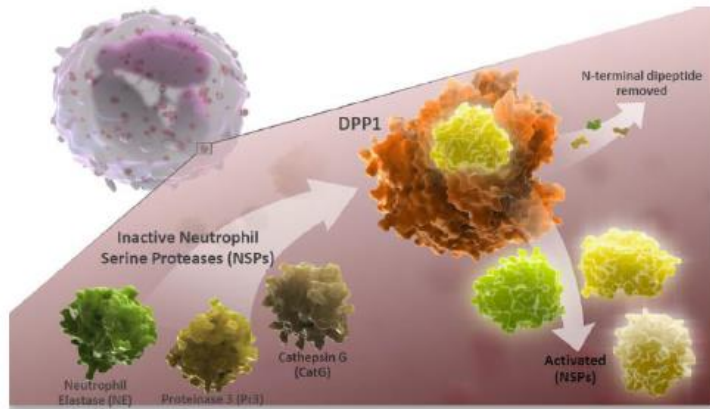
Etudes non académiques non spécifiques à la DCP

- Etude Aspen (laboratoire Insmed)
 - Efficacité d'un antiprotéase (brensocatib) dans la diminution des exacerbations
 - Etude en double aveugle sur un an
 - 10 centres français pneumologie adulte, un centre pédiatrique Creteil
 - Fin des inclusions 1500 adultes
- Etude AirLEAFTM (laboratoire BI)
 - Efficacité d'un antiprotéase (brensocatib) dans la diminution des exacerbations
 - Etude en double aveugle sur un an
 - Pas encore démarrée en France
 - Seulement adultes

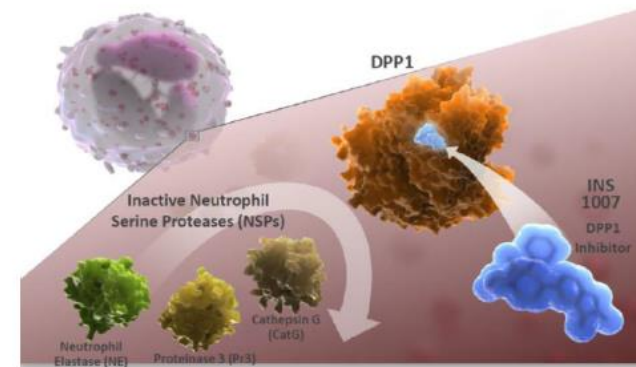
Rationnel de ses études sur les anti protéases



Neutrophil serine proteases (NSPs) are activated by dipeptidyl peptidase 1 (DPP1) during neutrophil maturation in bone marrow



Brensocatib inhibits DPP1, preventing activation of NSPs; neutrophils mature and are released without active NSPs





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BEAT-PCD ERS CRC 2023-2026 Management Committee


WP2  Suzanne

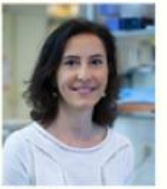
WP3  Jobst


WP4  Claire


WP5  Jane


WP6  Jean-François


 Hannah


 Marie


 Laura


 Claudia


 Susana

 Lidia


 Eric


 June


 Bernard


 Johanna


Chairs


 Lucy


 Trini


 Amelia


 Myrona


WP7  Claire


WP8  Guillaume


WP9  Yin Ting


 Katie


 Panayiotis

 Nisreen

 Mathieu

 Nora

 Nagehan

 Mieke



Principaux changements

- **WP1: Project management and governance**
- **WP2: Network of PCD research databases and collaborations**
- **WP3: Patient engagement activities**
- **WP5: Clinical outcome measurements**
- **WP7: Improving PCD diagnosis**
- **WP8: Improvement of clinical patient care (clinical standardisation and education)**
- **WP9: Engagement with the ERS and dissemination of CRC activities and results**

- **+ WP4 Biomarqueurs and bio ressources**
- **+ WP6 Neglected area for PCD research**

BEAT PCD

- Renouvellement
- **”identify the current knowledge gaps and research priorities for research in the field of PCD” questionnaire pour les soignants**

Clinical Trial Network

- Au départ ERN core (H Omran)
- Dir K Nielsen Codirecteurs B Maitre/J Raidt
- Interface entre centres européens rompus aux essais cliniques
Et les entreprises de la santé

Créer réseau de centres pouvant participer à des essais cliniques

Relecture/ commentaire des protocoles (A Coste, D Destouches)

Etudes de faisabilité ...

- 2 centres en France adulte/enfant : Créteil/Trousseau et Lyon

