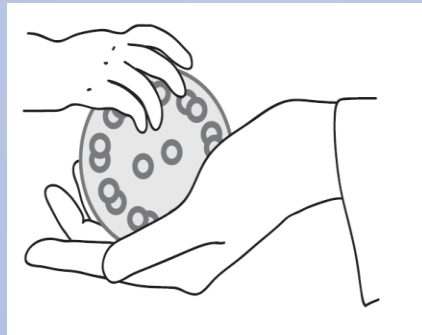


BESTCILIA

Better Experimental Screening and Treatment for
Primary Ciliary Dyskinesia



Bestcilia

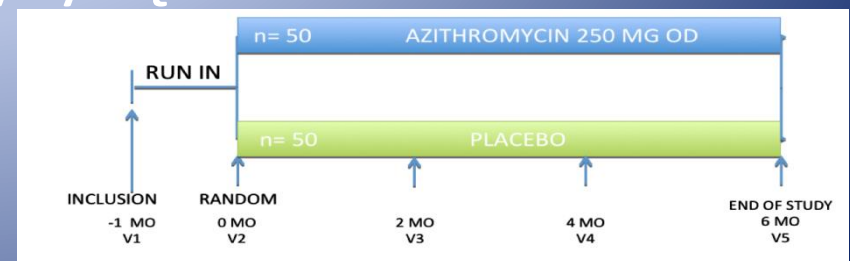
- Rozpoczęty 3 lata temu
- 7 PR UE
- Współpraca z prof. Heymutem Omranem
- konsorcjum

Konsorcjum

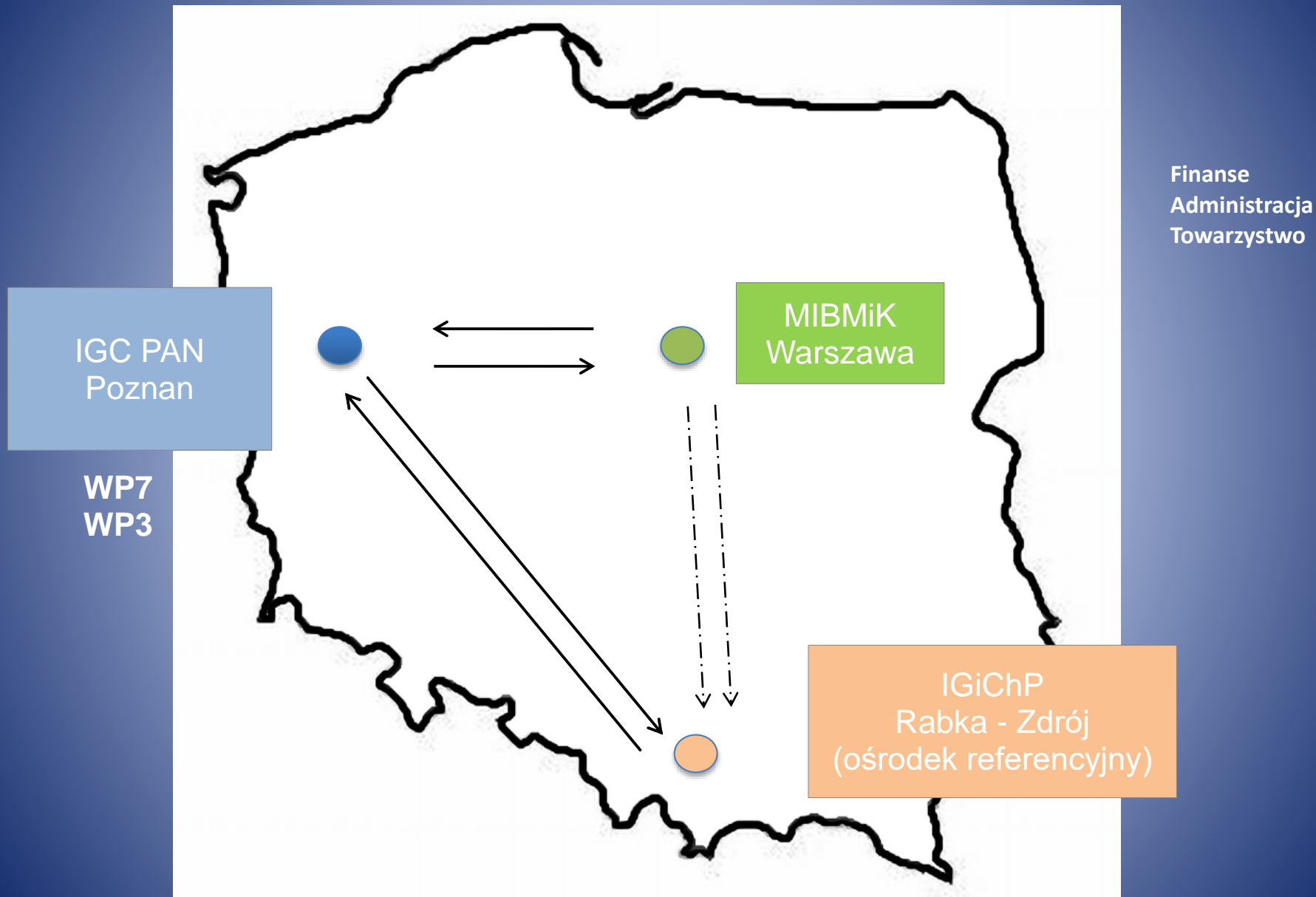
no.	Participant legal name	short name	Country	Organisation type
1	Westfälische Wilhelms-Universität Münster	WWU	DE	University (hospital)
2	University of Bern	UBERN	CH	University
3	Cyprus University of Technology	CUT	CY	University
4	University of North Carolina	UNC	USA	University (hospital)
5	Copenhagen University Hospital, Rigshospitalet	Region H	DK	University (hospital)
6	VU University Medical Center Amsterdam	VUMC	NL	University (hospital)
7	University of Southampton	SOTON	UK	University (hospital)
8	University of Miami	UM	USA	University (hospital)
9	International Institute of Molecular and Cell Biology, Warsaw	IIMCB	PL	Research Institute
10	University of Athens	UA	GR	University (hospital)
11	European Research Services GmbH	ERS	DE	Consultancy
12	Royal Brompton and Harefield National Health Service Trust	RBH	UK	University (hospital)

Główne cele

- Badania obserwacyjne charakteryzujące europejskich chorych na PCD - metaanaliza
- Ustanowić bazę danych do prospektywnej zbiórki danych o chorych (występowanie, objawy, leczenie przebieg)
- Wprowadzenie europejskiego standardu diagnostycznego (Cypr, Grecja, Polska)
- Opracowanie europejskiego kwestionariusza badania jakości życia
- Próba kliniczna z azytromycyną



Organizacja pracy



Od maja 2013

WP3 – nowoczesna diagnostyka

- Zakup aparatury do badania stęż. tlenku azotu w nosie (Ecomedics CLD88, NIOX MINO), kamera video, SAVA system
- Wypracowanie dokładnej standaryzowanej instrukcji prowadzenia badań
- Konsultacje uzyskanych wyników i obrazów
- Przebadanie grupy pacjentów z lat poprzednich oraz nowych
- Porównanie wyników – czy nowe metody coś dają?

WP7 - upowszechnienie

- Strona internetowa
- Broszura (6 języków, 7 krajów)
- Szkolenia wewnętrzne (3, w tym Kraków)
- Warsztaty specjalistyczne dla klinicystów (2, jw.)
- Upowszechnienie: EUREK Alert, AlphaGalileo)



Kraków 2013

BESTCILIA



NEWS

- Update in WP1
- Update in WP5
- Progress in WP2
- 20-23 of March 2014: PCD workshop/Bestcilia report session
- Progress in WP4
- WP3 update
- New Year wishes
- BESTCILIA: PCD diagnostic workshop 11 to 12 July 2013 Münster, Germany
- Bestcilia consortium meeting, 23 of November 2013, Naples, Italy
- Kick-off Meeting in Limassol, Cyprus

EVENTS

- Bestcilia meeting/PCD workshop** 20 to 23 March 2014 in Athens, Greece
- ERS School Course on "Primary ciliary dyskinesia: sharing knowledge and experience across Europe"** 21 to 23 November 2013, Naples, Italy
- ERS annual congress** 7 to 11 September 2013, Barcelona, Spain
- Bestcilia consortium meeting** 23 November 2013, Naples, Italy

PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

New Year wishes

We wish you



At the beginning of the New Year, we are starting a cycle of short updates on the progress within Bestcilia workpackages (WP). Today, information on WP3.

Realization of Work Package 3 (WP3) is following the schedule. WP3 aims to establish standardized PCD diagnostic testing in three European countries with limited health expenditures. During last year, Bestcilia WP3 participants have worked hard and significantly progressed in all areas necessary for achieving this goal. In few months, PCD patients from Cyprus, Greece and Poland will be able to get a reliable diagnosis of their condition, consistent with ERS diagnostic guidelines.

Establishing standardized PCD diagnostics in Cyprus, Greece and Poland will be achieved through establishment of national PCD diagnostic centers, development and introduction of standardized operational procedures (SOPs) and diagnostic algorithms for diagnostic PCD testing. During last year, Bestcilia participants from these three countries have significantly progressed in all areas necessary to achieve these goals (purchase and setting of equipment, receiving approval of local Ethics Committees, SOPs development and staff training).

The last goal was reached through the training workshop held at the University of Münster in Germany. The workshop dealt with the basic diagnostic methods for PCD: electron microscopy (EM), high speed video microscopy (HSV/M) and nasal nitric oxide (nNO) measurements, as well as the implementation of immunofluorescence techniques for confirming diagnostic results. The workshop was extremely successful, and apart from participants from Cyprus, Greece and Poland, physicians and researchers from other Bestcilia-participating countries were also present.

Based on this success, it was decided to organize a second workshop on that subject, open for all BESTCILIA and ERS PCD Task Force members. The workshop will take place in March 2014 in Athens, Greece. More information on this issue will follow soon.

Moreover, Bestcilia members have intensively discussed the details of SOPs and diagnostic algorithms to be implemented in PCD centers in Cyprus, Greece and Poland. The SOPs for nNO measurements are almost completed and SOPs for other PCD diagnostic methods are still being discussed. Therefore, within few months, suspect patients will be able to visit PCD centers in their country and get a reliable diagnosis consistent with ERS diagnostic guidelines.

Contact | Imprint | News | Events | Links

PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

Update in WP1

Datasets from more than 2000 PCD patients identified! (Progress in Work Package 1-WP1)

WP1 has been progressing according to plan. Its goal is to analyze existing clinical and epidemiological PCD datasets. This will allow to generate first representative data on clinical presentation, growth and development in PCD patients, as well as information on treatment effects and long-term prognosis for this orphan lung disease. So far, datasets from more than 2000 PCD patients have been identified and will soon be ready for analyses.

In a first step, existing PCD datasets have been identified by performing a systematic electronic search on published studies and conference abstracts. Unpublished datasets from members of the European Respiratory Society (ERS) PCD taskforce and other researchers have then been added to the list. So far, datasets for more than 2000 PCD patients have been identified and are potentially available for analyses.

In parallel, UNIBE has created the infrastructure necessary for the study by setting up data ownership, transfer and publication agreements, which leave all rights with data contributors. Furthermore, a safe IT platform for uploading anonymised data has been set up, and a precise description of desired variables has been compiled.

Several datasets have already been received and the process of cleaning, standardising and pooling of the data has begun. After the standardisation, the datasets will be available for first analyses. In the near future, individual patient data meta-analysis characteristics of the patient populations, lung function and growth.

PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

BESTCILIA: PCD diagnostic workshop 11 to 12 July 2013 Münster, Germany

In July 2013, Bestcilia partners have met in Münster, Germany, for an internal training on PCD diagnostics. The training was directed to participants of the workpackage 3 (introduction of Standardized Diagnostic PCD Testing in European Countries with limited Health Expenditures), but also other partners participated in this meeting.

At first, the general diagnostic algorithm for PCD was presented. Later lectures concerned application of different methods of detecting and confirming PCD, such as nasal NO measurement, transmission electron microscopy, immunofluorescent staining and identification of PCD-causative mutations. Meeting consisted both of lectures and practical hands-on training, moreover, participants exchanged their experiences in use of various diagnostic methods. During the meeting, a number of general issues were discussed.

At the end of the training, participants agreed to meet on 23rd of November 2013 in Naples, Italy.

PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

Update in WP5

AZITHROMYIN TRIAL IN PCD (WP5): PATIENTS' RECRUITMENT WILL START SOON! (Progress in Work Package 5)

Azithromycin is a widely used macrolide antibiotic, used for treatment of infection and as maintenance therapy in some chronic respiratory diseases. The aim of Bestcilia Work Package 5 (WP5) is to evaluate the efficacy and safety of azithromycin as maintenance therapy in PCD patients, which has so far not been assessed. This will be done through a randomized, placebo-controlled trial. We are delighted to inform that preparations for the trial have almost been finished and patient recruitment will start in spring 2014.

Azithromycin is an authorized drug widely used to treat infections and used as maintenance therapy in some chronic respiratory diseases, including PCD. However, the efficacy and safety of the use of this macrolide antibiotic as maintenance therapy in PCD patients, has not yet been evaluated. Such scientific, randomized, placebo-controlled trial of azithromycin is the aim of Bestcilia WP5.

This multi-national initiative will be conducted by Bestcilia partners from six institutions and five countries: Westfälische Wilhelms-Universität, Münster (Germany), University of Bern (Switzerland), VU University Medical Center Amsterdam (The Netherlands), University of Southampton (United Kingdom), Royal Brompton Hospital (United Kingdom) and Copenhagen University Hospital Rigshospitalet (Denmark).

In the past year the partners have been working intensively on the design of the trial and are now in the process of obtaining the required approval from health authorities and ethics committees in the participating countries.

A special workshop in "Nitrogen Multiple Breath Washout" was organized in November 2013 in Copenhagen in order to ensure standardization in the measurement and analysis of ventilation inhomogeneity between the participating centres. The "Lung Clearance Index" (LCI), which is one of the outcomes that can be obtained by Nitrogen Multiple Breath Washout, is one of the new spirometric outcome measures of the trial.

Recruitment of the 125 PCD patients for the trial is planned to begin in spring 2014.



Progress in WP2

PCD registry (WP2) is up and running!

International registry of PCD patients, which is the aim of Bestcilia Work Package 2 (WP2), will be ready for data entry from January 2014. The registry will allow systematic collection of data on incidence, clinical presentation, treatment and course of PCD. All the preparatory work has been done and the recruitment of patient will start soon.

During last year, partners involved in WP2 have been working intensively on the generation of the PCD registry. During and beyond the duration of Bestcilia project, the registry will be a wonderful instrument that will show some general trends in the clinical presentation, diagnostic accuracy and applied treatments in PCD. It will also allow to examine changes in long-term outcomes and recruit patients for multicentre clinical studies.

In the first step of the work, WWU has proposed potential items (patient parameters), which will be anonymously collected in the registry. ERS PCD Task Force experts and patient groups from Germany, Netherlands and USA had a significant role in this step. The potential parameters were later discussed by the partners from WWU, UBERNN, UNC, SOTON and Region H to include items, which are most universal/almost informative. Care has been taken to include parameters allowing comparisons with other datasets collected at present (GIS PCD registry) and in the past (for example with datasets generated during Bestcilia WP1).

PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

Progress in WP4

Today another update on the progress within Bestcilia:

PCD-SPECIFIC QUALITY OF LIFE QUESTIONNAIRES (WP4) IN ENGLISH ARE READY!

Work Package 4 (WP4) has progressed extremely well this year. The aim of WP4 is to develop and validate PCD-specific health related quality of life questionnaires (QOL-PCD). The questionnaires have been developed in English and translated into a number of languages, ready for validation.

Bestcilia partners from SOTON, UNC and UM developed the PCD-specific health related quality of life questionnaires (QOL-PCD, workpackage 4), with significant support from UNC, University College Cork, Royal Brompton Hospital. Participation of members of PCD Family Support Group UK and PCD Foundation USA has been vital to this study.

PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

13, the 4 prototype parents of child IL-PCD followed a 6 g FDA and European reviews with a large variation between lesion to be applied, culture and use of English into German development (complicated procedures will be easier).

Isolated versions of wing patients participated asked to complete forms. As soon as this also to other languages internationally.

Treatment for Prir

PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

Bestcilia investigator awarded with a grant!

GRANT FOR BESTCILIA INVESTIGATOR TO STUDY NOVEL METHODS OF DIAGNOSING PCD!

We are proud to inform you that Bestcilia investigator, Claire Hoggis team (Royal Brompton and Harefield NHS Foundation Trust, London, UK), have received a 6-year Health-care Scientist Post-Doctoral Fellowship from the National Institute of Health Research (NIH). Starting from April 2014, the lead scientist Amelia Shoemark who has won this award, will test novel methods of diagnosing PCD. If proved successful, they hope to significantly improve PCD diagnostics!

Primary Ciliary Dyskinesia (PCD) is diagnosed by the examination of cells using light and electron microscopy in 15-30% of children who have PCD, the cells look normal, and false negative results can delay diagnosis and the commencement of appropriate therapies. The purpose of this NIHR funded project is to test the use of novel approaches using 3D electron tomography to better define the ultrastructural defects that cause PCD. Increasing our understanding of ciliary ultrastructure and function aims to reduce diagnostic dilemmas and accelerate the diagnosis by improving our pick up rate and accuracy.

Amelia Shoemark, the lead scientist for the diagnostic service, and her team have already tried modern approaches to the analysis of ciliary structure. In their work published last year in Thorax, they visualized cilia by electron tomography (3D electron microscopy), finding significant differences in the ultrastructure of cilia that had previously been undetectable using current standard electron microscopy.

Now, thanks to the funding from the National Institute of Health Research, the group will be able to determine the usefulness of this and other novel techniques on a large number of patients. This study proves successful, this research team might significantly accelerate the diagnosis of PCD by improving the effectiveness of cilia visualization.

PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

Bestcilia publication about BMI and spirometry in PCD patients

Bestcilia publication: Relation of body mass index, lung function and sputum microbiology in PCD

Bestcilia investigators, members of the Advisory Board and collaborating teams have joined forces to perform a longitudinal study of the relationship between the body mass index (BMI), spirometry and sputum pathogens in PCD patients. Thanks to this collaboration, a group of over 150 PCD patients attending PCD referral centers in 3 countries has been analyzed. In agreement with the hypothesis, the results have shown that in PCD subjects receiving centralized care, nutrition and spirometric indices remain stable during medium term follow-up. Furthermore, BMI seems to be completely normal in PCD patients.

More information and the link to the article full text can be found [here](#)

PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

21-23 of March 2014: PCD workshop/Bestcilia report session

We would like to warmly invite you to the **International Conference on Primary Ciliary Dyskinesia (PCD)**, which will take place from 20 to 22nd of March 2014 in Athens, Greece.

The program of the conference will include lectures of the internationally recognized PCD experts and a specialist workshop covering all aspects of state-of-the-art PCD diagnostics. In addition, in parallel to the workshop, you will be able to participate in an open-to-public research meeting of Bestcilia participants.

Participation in our conference is free-of-charge and everybody is welcome to participate. Please see below for the details of the program and registration.

We hope you will find the program interesting and we are looking forward to see you on our conference!

INVITATION FOR THE MEETING (PDF, 0.3 MB)

HOTEL BOOKING FORM (Word, 0.8 MB)

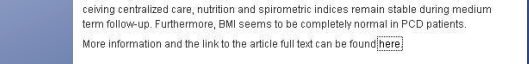
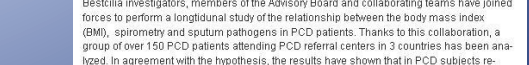
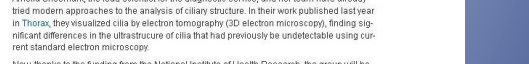
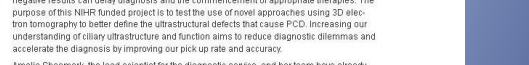
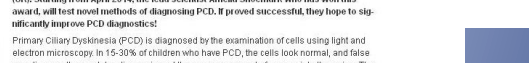
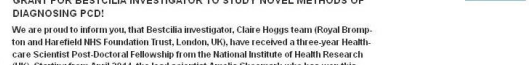
PROJECT ACTIVITIES CONSORTIUM WHAT IS PCD?

Bestcilia consortium meeting, 23 of November 2013, Naples, Italy

On 23rd of November 2013, Bestcilia participants have met in Naples, Italy. During the meeting, the leaders of each workpackage have presented the progress of work within each workpackage and discussed work schedule for the nearest future.

Apart from reporting the work, during the meeting, a short workshop on the health-related quality-of-life questionnaires (HRQOLQ) took place. The workshop was led by an external speaker, Alexandra L. Quittner (University of Miami, USA), who, together with Laura Behan and Jane Lucas (University of Southampton, UK) have presented the design and optimization of HRQOLQs in PCD. The presentations have been followed by discussions with all participants on the use of the HRQOLQ in clinical trials.

A number of general issues was also discussed, including publications and other dissemination activities. Participants have also debated on the organization of the future meeting in Athens, Greece. It has been decided that the meeting will take place from 21 to 23 March 2014 and will be connected with a specialized workshop for clinicians on PCD diagnostics.



BESTCILIA



NEWS

- Update in Work Package 2
- New publication by Bestcilia partner: further insight into PCD through a study on the Irish Traveller population
- New Bestcilia publication: new quality-of-life measure for adult PCD patients
- Update in WP 5
- Update in Work Package 3
- Update in WP4
- Huge success of the first Young Researchers Meeting in Bern!
- Update in Work Package 1
- Easter wishes
- New Bestcilia article: How does lobectomy

PROJECT

ACTIVITIES

CONSORTIUM

W

What is PCD?



Was ist Primäre Ziliäre Dyskinesie?
(PDF, 0.2 MB) (ZIP, 0.2 MB)



What is PCD?
(PDF, 1.7 MB) (ZIP, 1.7 MB)



Wat is Primaire Ciliaire Dyskinesie?
(PDF, 7.7 MB) (ZIP, 7.7 MB)



Τι είναι η ΠΔΚ - Πληροφορίες για ασθενείς
(PDF, 1.5 MB) (ZIP, 1.5 MB)



Τι είναι η ΠΔΚ - Πληροφορίες για ασθενείς
(PDF, 1.5 MB) (ZIP, 1.5 MB)



CZYM JEST PCD?
(PDF, 1.5 MB) (ZIP, 1.5 MB)



Hyad er PCD?

| CZYM JEST PCD?

Broszura informacyjna dla pacjentów

Autorami niniejszego tekstu są polscy uczestnicy programu Bestcilia: dr Zuzanna Bukowy-Bieryłło, prof. Michał Witt, prof. Ewa Ziętkiewicz, prof. Henryk Mazurek, dr Andrzej Pogorzelski.

Tekst częściowo został przygotowany w oparciu o materiały o PCD dostępne na stronie internetowej PCD Family Support Group w Wielkiej Brytanii (www.pcdsupport.org.uk), jak również broszurę przygotowaną przez niemieckich członków programu Bestcilia: dr Julię Wallmeier, dr Claudiusa Wernera i prof. Heymuta Omrana z Klinik für Kinder-und Jugendmedizin, Universitätsklinikum Münster w Niemczech.

W procesie tworzenia tej broszury, lekarze uwzględnili różnice regionalne oraz kulturowe w sposobach opieki nad pacjentami z PCD. Należy wziąć pod uwagę, że w chwili obecnej nie ma

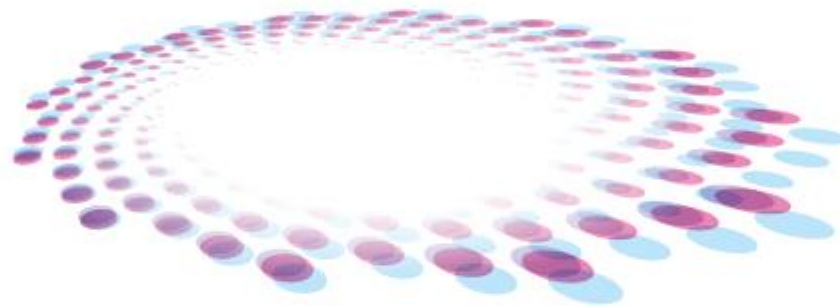


**Czym jest
Pierwotna Dyskineza Rzęsek?**

Broszura informacyjna dla pacjentów

Dzisiejsze spotkanie

- Organizacja spotkania – p. Agnieszka Ziemka
- Warsztaty szkoleniowe
- Reaktywacja Towarzystwa



**POLSKIE TOWARZYSTWO
DYSKINEZY RZĘSEK**