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Parent-reported quality of life measures for young children with primary ciliary dyskinesia

Submission date	**Recruitment status**	[_] Prospectively registered
27/05/2020	No longer recruiting	[_] Protocol
Registration date	**Overall study status**	[_] Statistical analysis plan
06/09/2022	Completed	[_] Results
Last Edited	**Condition category**	[_] Individual participant data
20/01/2025	Genetic Diseases	[X] Record updated in last year

Plain English summary of protocol

Background and study aims

In the airway, cells are lined with many hair-like structures called cilia that work together to sweep away and clear mucus, bacteria and otherdebris from the lungs, nose and ears. In primary ciliary dyskinesia (PCD), problems with the movement of the cilia result in mucus build-up. This causes constant symptoms from birth, which become worse during frequent chest, ear and sinus infections. All children with PCD eventually suffer permanent lung damage. PCD occurs in about 1 in 10,000 people and is caused by a genetic change inherited from both parents.

There is a need for new treatments to prevent children with PCD from getting infections and to delay lung damage. Treatments are also needed to improve how children with PCD feel in their daily life in terms of reducing symptoms and improving their energy levels. Researchers are developing new treatments and now urgently need ways to measure whether these treatments work. Health-related quality of life questionnaires provide a way for patients to report changes in their symptoms and well-being when they start a new treatment. Patients are more likely to stick with a treatment if it makes them feel and function better or if it has fewer side effects. Young children may be unable to explain how they feel, so instead, their parents will be asked about their child's symptoms and how these affect daily living such as sleeping and eating. Asking the child's parent about how their child's disease affects them at a particular time in a standard way also helps to measure the impact of a new treatment.

The aim of this study is to find out the symptoms and burdens that are most important to the child and parent and to create and test a questionnaire that asks about these symptoms in a standard way. This questionnaire will provide a way for patients to report changes in their child's symptoms and well-being when they start a new treatment.

Who can participate?

Parents of children with primary ciliary dyskinesia aged 6 years or below.

What does the study involve?

The researchers have already developed health-related quality of life questionnaires for older children and adults (called QOL-PCD) that are being used to decide whether treatments work in patients with PCD. These questionnaires have been translated into many languages and are being used in research studies across the world. the research team will use this expertise to develop parent-reported questionnaires for younger children. A researcher will interview 20-30

parents to understand how PCD impacts their child's life. They will use this knowledge to develop a questionnaire called QOL-PCDPR (Parent-Reported). The questionnaires will be tested by 70 parents, to ensure it is a strong and accurate measure for testing whether a particular treatment works in pre-school children with PCD.

What are the possible benefits and risks of participating?
The benefits in participating in this study is that it provides parents of very young children an opportunity to provide their voice and enable their experiences to be incorporated into the parent-reported questionnaire.
The are no risks in participating in this study.

Where is the study run from?
The University of Southampton (UK)

When is the study starting and how long is it expected to run for?
October 2019 to December 2023

Who is funding the study?
National Institute for Health Research (NIHR) (UK)

Who is the main contact?
l.behan@soton.ac.uk

Contact information

Type(s)
Public

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Additional identifiers

Clinical Trials Information System (CTIS)
Nil known

Integrated Research Application System (IRAS)
63800

ClinicalTrials.gov (NCT)
Nil known

Protocol serial number
NIHR200470, IRAS 63800

Study information

Scientific Title
Parent-reported Quality of Life measures for young children with Primary Ciliary Dyskinesia (QOL-PCD)

Acronym
QOL-PCD

Study objectives
To develop and validate a parent-reported outcome measure to evaluate the impact of PCD in young children: QOL-PCDPR. The QOL-PCDPR will eventually be used for monitoring in clinical practice and for use as an outcome measure in clinical trials.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Approved 27/09/2019, NHS Health Research Authority South Central - Hampshire A Research Ethics Committee (Level 3, Block B, Whitefriars, Lewins Mead, Bristol, BS1 2NT, UK; +44 (0)207 104 8214; hampshirea.rec@hra.nhs.uk), ref: 06/Q1702/109

Study design
Observational cross sectional international multi center study

Primary study design
Observational

Study type(s)
Quality of life

Health condition(s) or problem(s) studied
Primary ciliary dyskinesia

Interventions
To inform the development of the patient-reported outcome measure (QOL-PCDPR), individual, semi-structured open-ended interviews will be conducted by telephone with parents by the research fellows who have extensive training and experience in conducting qualitative interviews and has no pre-existing relationships with the study participants. Interviews will be audio-taped and transcribed using content analysis using NVivo (version 8.0, QSR International Pty Ltd). Elements of the coding and analyses will independently conducted by the two Research Fellows who then reach consensus. Thematic coding will identify key symptoms and impacts. These data will be analyzed for their frequency of endorsement and level of impact. Saturation matrices will inform item generation to ensure that data saturation is achieved (i.e. no new themes arising with new interviewees). Agreement on question selection for the questionnaire and wording will be agreed during multi-disciplinary, multinational conference calls using a modified Delphi approach; we will discuss the specific quotes and saturation grids from the interviews. Selected items will be written using parent language as used in the qualitative interviews; the questions will then be combined into subscales based on the research team's conceptual framework.

Participants will also be asked to complete questionnaires assessing quality of life to support the validation of the QOL-PCDPR.

Intervention Type
Other

Primary outcome(s)
1. Parental burden assessed using a single semi-structured interview by telephone
2. Parent's assessment of the child's quality of life at a single time point:
2.1. The Infant Toddler Quality of Life Questionnaire (47 item short form)
2.2. The Parent Cough-Specific Quality of Life (8 item short form)
2.3. Sinunasal Questionnaire (SN-5)
2.4. Otitis media-6 questionnaire
2.5. The prototype QOL-PCDPR questionnaire (for validation)

Key secondary outcome(s))
There are no secondary outcome measures.

Completion date
14/12/2023

Eligibility

Key inclusion criteria
Parents or guardians of young children (aged 0-6 years) who have received a diagnosis of primary ciliary dyskinesia

Participant type(s)
Carer

Healthy volunteers allowed
No

Age group
Adult

Sex
All

Key exclusion criteria
Does not meet inclusion criteria

Date of first enrolment
01/11/2019

Date of final enrolment
30/09/2023

Locations

Countries of recruitment
United Kingdom

England

Australia

Canada

United States of America

Study participating centre
University Hospital Southampton
University Hospital Southampton NHS Foundation Trust
Tremona Road
Southampton
United Kingdom
S016 6YD

Study participating centre
The Leeds Teaching Hospitals NHS Trust
Great George St
Leeds
United Kingdom
LS1 3EX,

Study participating centre
University Hospitals of Leicester NHS Trust
Infirmary Square
Leicester
United Kingdom
LE1 5WW

Study participating centre
Royal Brompton and Harefield NHS Foundation Trust
Britten St
Chelsea
London
United Kingdom
SW3 6NJ

Study participating centre
Hospital for Sick Children (SickKids)
555 University Ave
Toronto
Canada
ON M5G 1X8

Study participating centre
UNC Healthcare
North Carolina
Chapel Hill
United Kingdom
ON M5G 1X8

Study participating centre
The Royal Children's Hospital
50 Flemington Rd
Parkville

Melbourne
Australia
3052

Sponsor information

Organisation
University Hospital Southampton NHS Foundation Trust

ROR
https://ror.org/0485axj58

Funder(s)

Funder type
Government

Funder Name
National Institute for Health Research

Alternative Name(s)
National Institute for Health Research, NIHR Research, NIHRresearch, NIHR - National Institute for Health Research, NIHR (The National Institute for Health and Care Research), NIHR

Funding Body Type
Government organisation

Funding Body Subtype
National government

Location
United Kingdom

Results and Publications

Individual participant data (IPD) sharing plan
The datasets generated during the current study will be available upon request from Jane Lucas (jlucas1@soton.ac.uk) and Laura Behan (l.behan@soton.ac.uk). The type of data will include qualitative data generated through semi-structured interviews with all identifiers removed. It will be available from the publication of findings in a scientific journal and in accordance with R&D protocols.

IPD sharing plan summary

Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Participant information sheet	Participant information sheet	11/11/2025	11/11/2025	No	Yes