

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

Submission date 27/05/2020	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered <input type="checkbox"/> Protocol
Registration date 06/09/2022	Overall study status Completed	<input type="checkbox"/> Statistical analysis plan <input type="checkbox"/> Results
Last Edited 20/01/2025	Condition category Genetic Diseases	<input type="checkbox"/> Individual participant data <input checked="" type="checkbox"/> 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 other debris 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.

There 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

EudraCT/CTIS number

Nil known

IRAS number

63800

ClinicalTrials.gov number

Nil known

Secondary identifying numbers

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

Secondary study design

Mixed methods study involving both qualitative and quantitative methods to determine what factors impact on parents and very young children with PCD. Data will be analysed to develop and validate a parent-reported outcome measure.

Study setting(s)

Community

Study type(s)

Quality of life

Participant information sheet

Not available in web format, please use contact details to request a participant information sheet

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 measure

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)

Secondary outcome measures

There are no secondary outcome measures.

Overall study start date

01/10/2019

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

Age group

Adult

Sex

Both

Target number of participants

70 participants

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

Australia

Canada

England

United Kingdom

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

Infirmery 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

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SO166YD
+44 (0)23 8077 7222
sponsor@uhs.nhs.uk

Sponsor type

Hospital/treatment centre

Website

<http://www.uhs.nhs.uk/home.aspx>

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

Publication and dissemination plan

An objective of this study is to prepare outputs, including the report for NIHR, manuscripts for publication, abstracts for conferences, information for PCD Support Group website and social media.

In particular, to ensure that the QOL-PCD is freely and widely available, whilst remaining within the scope of the validation, we have copyright for the existing versions. The researchers have informed the PCD community, through international networks and publications, how to gain approval for free use for clinical and research purposes. Applicants provide four clinical researchers who developed QOL-PCD with a short protocol; they sign a declaration that they will administer, score and report the QOL-PCD according to the instruction manual. The researchers plan to make this an online procedure by the time the QOL-PCDPR is ready for use, hosted by University of Southampton.

Participation by PCD-clinical leads from other English speaking countries (Australia, Canada, USA) will ensure rapid dissemination for clinical use. The researchers' previous studies for QOL-PCD have resulted in publications in European Respiratory Journal, Thorax and Annals of American Thorax Society and they would target these journals again. The researchers aim to present the QOL-PCDPR at the leading North American and European Respiratory meetings (ATS and ERS). They will use the global network BEAT-PCD to further disseminate their activities using social media and websites. The PCD Support group will ensure patients are informed of the study and its outputs through presentations by researchers at meetings, via social media, the website and newsletter.

Intention to publish date

14/12/2024

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