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A study on the impact of the effect of mobile health on airway clearance in patients with primary ciliary dyskinesia

Submission date 08/10/2024	**Recruitment status** No longer recruiting	[_] Prospectively registered [X] Protocol
Registration date 24/10/2024	**Overall study status** Completed	[_] Statistical analysis plan [_] Results
Last Edited 09/10/2024	**Condition category** Respiratory	[_] Individual participant data [X] Record updated in last year

Plain English summary of protocol

Background and study aims

Primary Ciliary Dyskinesia (PCD) is a rare genetic condition where the cilia, hair-like structures lining the airways, do not function properly. Normally, cilia move mucus and trapped pollutants out of the airways, but in PCD, this process is impaired, leading to mucus buildup and recurrent respiratory infections. These infections can cause irreversible lung damage and affect the ears, sinuses, and lungs. PCD is also linked to sub/infertility, congenital heart defects, and organ laterality defects. There is no bespoke treatment for PCD. Current treatments focus on preventing infections and managing symptoms through Airway Clearance Techniques (ACTs) and antibiotics. Many different types of ACTs are used by patients, most of which involve repeated breathing exercises to move mucus through the airways. Some patients with PCD will use inhalers or nebulisers (e.g., hypertonic saline) before their ACT to loosen the mucus in the airways. Upper airway hygiene involves nasal rinsing with hypertonic saline and/or topical nasal corticosteroids and antibiotics to manage sinus and nasal symptoms. Regular exercise may be also recommended to increase mucus clearance and improve factors that can affect respiratory health (such as cardiovascular fitness, weight, and lung function). Good engagement with ACTs may result in improved respiratory outcomes. However, engaging in a daily routine of ACTs is time-consuming and may be burdensome for PCD patients, leading to performing less efficient techniques or ultimately discontinuing the routine. Furthermore, some PCD patients, particularly young patients, view the performance of ACTs as a visible sign of ‘being ill’ and may be reluctant to carry them out systematically. This study aims to show that a mobile health app and a wrist-worn wearable device can support PCD patients in their daily ACT routines, improving their quality of life and reducing exacerbations.

Who can participate?

Adolescents (from age 14 years old upwards) and adults diagnosed with PCD who are prescribed daily ACT routines

What does the study involve?
The study will use a mobile health app and a wrist-worn wearable device to support daily ACT routines. Engagement and wearable data will be collected to track exercise and physical activity. The impact on patients' quality of life and the occurrence of exacerbations will be assessed.

What are the possible benefits and risks of participating?
Benefits include improved engagement with ACT routines, better respiratory outcomes and quality of life, as well as reduced occurrence of exacerbations.

Risks are minimal, as the intervention involves non-invasive technology.

Where is the study run from?
Aparito Limited

When is the study starting and how long is it expected to run for?
October 2021 to May 2024

Who is funding the study?
1. Aparito Limited
2. PCD Support UK

Who is the main contact?
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Public

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Additional identifiers

EudraCT/CTIS number
Nil known

IRAS number

ClinicalTrials.gov number
Nil known

Secondary identifying numbers
Nil known

Study information

Scientific Title
Randomized controlled study to investigate the impact of mobile health (mobile app and wearables) on the engagement with prescribed airway clearance techniques in patients with primary ciliary dyskinesia (PCD)

Acronym
PCD-ENGAGE

Study objectives
It is hypothesized that the use of mobile health in the form of a bespoke patient-facing app and wearables with behaviour change features tailored to patients' preferences, may improve engagement with prescribed airway clearance daily routine in PCD patients, which may result in improved quality of life and reduced exacerbation occurrence.

Ethics approval required
Ethics approval required

Ethics approval(s)

Approved 30/11/2022, Reading Indepdent Ethics Committee (93 Reading Road, Woodley, Reading, RG5 3AE, United Kingdom; +44 (0)1189691022; m.l.arnott@btinternet.com), ref: None available

Study design
Fully decentralised randomized controlled trial

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)
Home

Study type(s)
Other, Quality of life

Participant information sheet
See study outputs table

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

Interventions
The study involves a behavioural change intervention via a mobile health app and wrist-worn wearables to support engagement with airway clearance techniques (ACT).

Participants were randomised via an algorithm built into the study platform (Atom5™) following screening, e-consent and eligibility confirmation.

Control group arm: Participants used the app to self-report their engagement with ACTs and only received notifications to prompt logging of requested ACT data regularly to ensure enough engagement with the technology to collect the study data. Participants were also prompted to complete some patient-reported outcomes (PROs) at baseline, 6 weeks, and 12 weeks. These were: QOL-PCD, Patient Health Engagement scale (PHQ), Exacerbation events questionnaire and Spirometry readings. The total follow-up time of patients within the study was 12 weeks. Once the 12-week study period was finished, all participants in the control arm were invited to voluntarily cross over to the intervention group, to receive the activity tracker and were followed for up to 24 weeks (including answering the PROs at weeks 18 and 24). Participants crossing over from the control group provided consent (and assent was applicable) to continue in the extension phase.

Intervention arm group: Same as above with the addition of a consumer-grade CE-marked Garmin Vivosmart® 5 wearable activity tracker. The wearable device was synchronised to the Atom5™ app and passively collected data such as heart rate (HR), activity levels (step count), motion type and intensity, time spent in moderate-to-vigorous activity, and sleep. This data was visualised in real time on a study-dedicated clinician dashboard.

This arm also included the following behaviour change features:
• Reminders - to sync the wearable, and log physiotherapy data
• Compliance reports to notify participants of weekly ACT engagement.
• Weekly physical activity reports to notify participants based on activity data collected through the Garmin watch.
• Rewards: bronze, silver and gold badges were to be issued upon reception of ACT e-diaries for 3, 7 and 14 days in a row, respectively.

Intervention Type
Behavioural

Primary outcome measure
Change in engagement with prescribed airway clearance techniques (ACTs) measured using data collected in a daily self-reported ACT diary to the end of the study period (week 12)

Secondary outcome measures
Health-related quality of life (HRQoL) of people with PCD measured using the PCD-specific QoL instrument: QOL-PCD adult and adolescent (13-17) versions at baseline, weeks 6, and 12. Additionally, weeks 16 and 24 for those participating in the extension.

Overall study start date
01/10/2021

Completion date
30/05/2024

Eligibility

Key inclusion criteria
1. Adolescents (from 14 upwards) and adults diagnosed with PCD (written proof such as a letter of diagnosis from their reference specialist centre will be required, and/or genetic information, if known) and performing regular airway clearance techniques (ACTs)
2. Able and willing to provide consent or, if appropriate, participants having an acceptable individual capable of providing consent on their behalf, e.g., parent or guardian of a child under 18 years of age
3. Able to speak and understand English
4. Able and willing to engage with the technology
5. Access to an internet-connected smartphone with iOS or Android system

Participant type(s)
Patient

Age group
Mixed

Lower age limit
14 Years

Upper age limit

100 Years

Sex
Both

Target number of participants
40

Total final enrolment
19

Key exclusion criteria
1. Severe/acute disease, including:
1.1. Severe haemoptysis 6 months prior study start
1.2. Congenital heart defects, other than dextrocardia
1.3. Cardiac disease
1.4. Ongoing exacerbation. Once their exacerbation is resolved, they could be considered eligible for the study.

Date of first enrolment
17/04/2023

Date of final enrolment
10/11/2023

Locations

Countries of recruitment
England

United Kingdom

Wales

Study participating centre
Aparito Limited
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Sponsor information

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Funder(s)

Funder type
Industry

Funder Name
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Funder Name
PCD Support UK

Results and Publications

Publication and dissemination plan
Planned publication in a peer-reviewed journal

Intention to publish date
01/01/2025

Individual participant data (IPD) sharing plan
The data sharing plans for the current study are unknown and will be made available at a later date

IPD sharing plan summary
Data sharing statement to be made available at a later date

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?

Participant information sheet	version 1.5	21/11/2023	09/10/2024	No	Yes
Protocol file	version 4.0	21/11/2023	09/10/2024	No	No