

Improvement of pain and quality of life in patients with sickle cell disease with overnight oxygen therapy or auto-adjusting positive airway pressure (APAP)

Submission date 03/06/2014	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered <input checked="" type="checkbox"/> Protocol
Registration date 18/07/2014	Overall study status Completed	<input type="checkbox"/> Statistical analysis plan <input checked="" type="checkbox"/> Results
Last Edited 04/03/2021	Condition category Haematological Disorders	<input type="checkbox"/> Individual participant data

Plain English summary of protocol

Background and study aims

Sickle cell disease (SCD) is an inherited blood disorder where the red blood cells do not develop normally. Instead of being flexible and disc-shaped like normal blood cells, they are rigid and shaped like a crescent (or sickle). They also contain defective haemoglobin, the iron-rich protein that enables red blood cells to carry oxygen. This results in lower than normal oxygen levels in the tissues and organs of the body and can cause people to feel lethargic (lack of energy), tired and breathless, particularly after exercise. Many complications of the condition are thought to be caused by low daytime and night time oxygen levels. These can be made worse if the patient also suffers from obstructive sleep apnoea (OSA) where the muscles and soft tissues in the windpipe relax and collapse during sleep, causing a total blockage of the airway; this results in an extra dip in oxygen levels during sleep. Overnight oxygen is already used in patients with chronic lung disease who suffer from low oxygen levels during sleep. Automatic positive airway pressure (APAP) machines, which work by blowing air at a pressure that keeps the windpipe open, are used successfully for OSA patients of all ages. It is possible that these treatments may also help people suffering from SCD. Early studies have shown that SCD patients do find overnight oxygen safe and easy to use. There is also some evidence that APAP can be beneficial to children with SCD, resulting in improved attention span and fewer crises. However, further work is needed to decide whether the inconvenience of these treatments outweigh any benefits from using them for treating dips in night time oxygen levels for SCD patients. The aim of this small scale (pilot) study is to find out which intervention (the overnight oxygen or APAP) is the most acceptable for patients by asking them to use them for one week each and then have an interview describing their experience. The most acceptable intervention will then be used for further study.

Who can participate?

English speaking SCD patients aged over 8 years and who are able to (or their parents are able to) use a smart phone.

What does the study involve?

Participants are first asked to fill in a daily pain diary for a week. They are then randomly allocated to receive one of the two interventions. A sleep physiotherapist sets up the intervention and shows the participant how to use it. The participant then uses the intervention for a week, filling in their pain diary daily and will have phone interviews to discuss any symptoms suffered. After the seven day treatment, the intervention is stopped and the participant attends hospital for a review, which includes having blood and urine tests taken, and having their lung function and oxygen levels measured. Afterwards, the participant continues to fill in their pain diary for another week before repeating the procedure with the second intervention.

What are the possible benefits and risks of participating?

Participants may find that their condition improves from one or both interventions given in this trial. If this proves to be the case, we will leave the machine that provides the most benefit with the patient (with approval from their doctor). As regards to risks, some people may find the tests to check lung function uncomfortable and may make them feel dizzy. Blood tests can also be slightly painful. Overnight APAP and oxygen therapies may also be uncomfortable and inconvenient to the participant. There is evidence that higher doses of oxygen can stop new red blood cells from being made, but not with the doses and timing of the oxygen being used for this study.

Where is the study run from?

University Hospital, Southampton (UK)
Guy's and St Thomas' NHS Foundation Trust (UK)
UCL Institute of Child Health (UK)

When is the study starting and how long is it expected to run for?

July 2014 to December 2014

Who is funding the study?

National Institute for Health Research (UK)

Who is the main contact?

Professor Fenella Kirkham
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Contact information

Type(s)

Scientific

Contact name

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Additional identifiers

EudraCT/CTIS number
2014-002181-67

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
RHMCHI0706

Study information

Scientific Title

Prevention Of Morbidity in Sickle cell disease 2a - pilot phase (POMS 2a): Improvement of pain and quality of life in patients with sickle cell disease with nocturnal oxygen therapy or auto-adjusting continuous positive pressure: pilot phase

Acronym

POMS

Study objectives

The principal research question is to assess whether APAP or overnight oxygen therapy is more acceptable to patients and to assess whether there are any physiological effects of either therapy. This information will influence the choice of intervention in a second, proof of concept trial.

Ethics approval required

Old ethics approval format

Ethics approval(s)

NRES Committee East of England - Cambridge South, 03/06/2014, ref: 14/EE/0163

Study design

Randomised controlled trial. Block randomisation by an independent statistician at RDS Southampton.

Primary study design

Interventional

Secondary study design

Randomised controlled trial

Study setting(s)

Hospital

Study type(s)

Quality of life

Participant information sheet

Not available in web format, please use the contact details below to request a patient information sheet

Health condition(s) or problem(s) studied

Sickle Cell Disease

Interventions

Participants will have two interventions, APAP and overnight oxygen, for a week each in randomized order (week 2 and 4). There will be a week of baseline data collection (week 1), and a week of washout between the interventions (week 3).

Intervention Type

Device

Primary outcome measure

Patient feasibility, acceptability and preference will be explored using qualitative interviews

Secondary outcome measures

1. Pain: Using available Smartphone technology, information on:
 - 1.1. Pain characteristics (intensity, location, quality)
 - 1.2. Pain medications and non-pharmacological strategies used for pain
 - 1.3. Healthcare visits will be collected in the pilot to test out the methodology and to determine whether there is any obvious effect of either intervention or its withdrawal, particularly if detrimental. This is important as the first outcome measure for trial 2b will be average pain intensity during the two observation periods.
2. Adverse events: The Clinical Report Forms (CRFs) for reporting adverse events will be trialed during this pilot to show efficacy in recording and reporting adverse events.
3. Daytime oxygen saturation will be collected before and after each intervention to determine whether there is any obvious effect of either intervention or its withdrawal, particularly if detrimental.
4. Lung volume will be collected before and after each intervention to determine whether there is any obvious effect of either intervention or its withdrawal, particularly if detrimental.

Overall study start date

01/07/2014

Completion date

01/12/2014

Eligibility

Key inclusion criteria

1. Age over 8 years
2. Informed consent with assent in accordance with the institutional policies (UK ethical committee) and European or US Federal guidelines
3. Sickle cell haemoglobin (HbSS) diagnosed by standard techniques

4. Able to speak and understand English
5. Patient or parent/guardian able to use smart phone

Participant type(s)

Patient

Age group

Adult

Sex

Both

Target number of participants

20

Total final enrolment

21

Key exclusion criteria

1. Patient already on overnight respiratory support or has used it in the past
2. Hospital admission for acute sickle complication within the past 1 month
3. Patient with > 6 admissions for acute sickle complications within the past 12 months
4. Existing Respiratory Failure
5. Decompensated Cardiac Failure
6. History of Severe Epistaxis
7. Transsphenoidal

Date of first enrolment

01/07/2014

Date of final enrolment

01/12/2014

Locations**Countries of recruitment**

England

United Kingdom

Study participating centre

University Hospital Southampton

Southampton

United Kingdom

SO16 6YD

Study participating centre

Guy's and St Thomas' NHS Foundation Trust

London

United Kingdom

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Study participating centre

UCL Institute of Child Health

London

United Kingdom

-

Sponsor information

Organisation

University Hospital Southampton NHS Foundation Trust (UK)

Sponsor details

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Sponsor type

Hospital/treatment centre

ROR

<https://ror.org/0485axj58>

Funder(s)

Funder type

Government

Funder Name

National Institute for Health Research (NIHR) (UK) - CCR- RfPB grant

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

Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Protocol article	protocol	25/08/2015		Yes	No
Results article	results	18/07/2019	04/03/2021	Yes	No
HRA research summary			28/06/2023	No	No