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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

Registration date
18/07/2014

Overall study status
Completed

Last Edited
04/03/2021

Condition category
Haematological Disorders

[ ] Prospectively registered
[X] Protocol
[ ] Statistical analysis plan
[X] Results
[ ] 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 are 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 them 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

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Additional identifiers

Clinical Trials Information System (CTIS)
2014-002181-67

Protocol serial number
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

Study type(s)
Quality of life

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

Key secondary outcome(s))
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.

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

Healthy volunteers allowed
No

Age group
Adult

Sex
All

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
United Kingdom

England

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
-

Study participating centre
UCL Institute of Child Health
London
United Kingdom
-

Sponsor information

Organisation
University Hospital Southampton NHS Foundation Trust (UK)

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

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?
Results article	results	18/07/2019	04/03/2021	Yes	No
Protocol article	protocol	25/08/2015		Yes	No
HRA research summary			28/06/2023	No	No
Participant information sheet	Participant information sheet	11/11/2025	11/11/2025	No	Yes