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Hydrotherapy in mucopolysaccharidosis II

Submission date
23/10/2023

Recruitment status
No longer recruiting

Registration date
25/10/2023

Overall study status
Completed

Last Edited
02/11/2023

Condition category
Nutritional, Metabolic, Endocrine

[ ] Prospectively registered
[ ] Protocol
[ ] Statistical analysis plan
[ ] Results
[ ] Individual participant data
[ ] Record updated in last year

Plain English summary of protocol

Background and study aims
Hydrotherapy (also known as aquatic therapy) is exercise therapy in warm water delivered by a physiotherapist. The unique properties of warm water can improve the movement of stiff or swollen joints, provide relaxation, strengthen weak muscles and alleviate aches and pains. Hydrotherapy has been shown to be particularly effective for pain in other conditions as aquatic buoyancy potentially reduces weight-bearing stresses on joints, bones and muscles. Hydrotherapy also allows the performance of closed-chain exercises, which are potentially painful with greater weight bearing when out of water. The pain syndromes seen in mucopolysaccharidosis type II (MPS) patients are complex and often multifactorial with elements of neuropathic pain, musculoskeletal pain and frequently a mixed picture with both. Patients with MPS present with joint, bone, and muscle pain. Non-surgical approaches such as physiotherapy have been advocated as benefitting patients with MPS II. Anecdotal evidence has supported the benefits of hydrotherapy delivered by a physiotherapist in the physiotherapy management of MPS disorders. It has been shown to decrease joint stiffness, reduce pain and improve strength. However, there is limited high-quality evidence currently to support its use as a routine part of care for this group of patients. The main aim of this study is to evaluate the effectiveness of hydrotherapy either positively or negatively on the quality of life of MPS II patients on long-term enzyme replacement therapy (ERT).

Who can participate?
Male patients aged 18-58 years with mucopolysaccharidosis type II

What does the study involve?
The hydrotherapy session will last 30 minutes in a heated pool and an experienced physiotherapist will deliver the programme and assessments. Patients will be asked to attend such sessions once a week for 12 weeks. Prior to the study, at 6 weeks into the study and at the end of 12 weeks patients will be asked to complete a patient satisfaction questionnaire. No blood samples will be collected apart from usual blood tests as part of the routine care in the metabolic clinic.

What are the possible benefits and risks of participating?
The study may have a direct benefit to MPS II patients and will help our understanding of the effectiveness of hydrotherapy in the management of joint pain, improved mobility and quality of life in adult patients with MPS type II. No serious risks have been identified. The rare but

possible risks include fatigue, trip/slip on entering the pool, and skin irritation. Water inhalation or drowning are risks of hydrotherapy in general.

Where is the study run from?
Salford Royal Organisation, Northern Care Alliance (UK)

When is the study starting and how long is it expected to run for?
June 2023 to October 2024

Who is funding the study?
MPS Society UK

Who is the main contact?
1. Karolina M Stepien, CI
2. Marie Meehan, Study Coordinator

Contact information

Type(s)
Scientific, Principal Investigator

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Additional identifiers

EudraCT/CTIS number

Nil known

IRAS number
317767

ClinicalTrials.gov number
Nil known

Secondary identifying numbers
22MET03-S, IRAS 317767, CPMS 56760

Study information

Scientific Title
Does hydrotherapy alleviate pain and improve functional mobility in patients with mucopolysaccharidosis type II (Hunter syndrome)?

Study objectives
Hydrotherapy is effective for pain as aquatic buoyancy potentially reduces weight-bearing stresses on joints, bones and muscles. It improves range of movement and reduces reliance on walking aids and the need for painkillers.

Ethics approval required
Ethics approval required

Ethics approval(s)
Approved 15/06/2023, North West - Greater Manchester South Research Ethics Committee (3rd Floor, Barlow House, 4 Minshull Street, Manchester, M1 3DZ, United Kingdom; +44 (0)207 104 8014; gmsouth.rec@hra.nhs.uk), ref: 23/NW/1069

Study design
Randomized cross over trial

Primary study design
Interventional

Secondary study design
Randomised cross over trial

Study setting(s)
Hospital

Study type(s)
Prevention, Quality of life, Efficacy

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

Mucopolysaccharidosis type II

Interventions
Participants are randomised into two groups A and B. While group A initially receives the hydrotherapy treatment, group B receives nothing and completes pain and quality of life questionnaires. Then there is a washout period. Then group B receives the treatment whilst group A receives no therapy but completes the questionnaires. The outcome is measured before each treatment and between the treatments. This study design will give 10 data points for analysis. The hydrotherapy lasts 12 weeks and the control arm will last 12 weeks. Overall each patient will participate in the study over 24 weeks.

Intervention Type
Other

Primary outcome measure
Quality of life assessed using the Short Form Health Survey 36 scale (performed by nurse) at baseline, 6 weeks and 12 weeks while undergoing hydrotherapy and then separately while in the non-intervention arm

Secondary outcome measures
Assessed at baseline, at a 6- and 12-week follow-up unless stated otherwise:
1. Risk assessment at baseline
2. Symptom severity assessed using the Brief Pain Inventory and pain-related questionnaires related to anxiety (GAD-7), quality of life (PHQ9) and kinesiophobia (TSK11) (performed by pain specialist/nurse)
3. The objective LANSS score will be used to identify and exclude patients who suffer from predominantly neuropathic pain (rather than musculoskeletal nociceptive-driven pain). This assessment will be performed by a pain specialist.
4. Global impression of change for patients with learning difficulties as assessed by the MDT team (consultant, physiotherapist & nurse)
5. Physiotherapy assessment including joint range of movement, muscle strength, functional mobility using ‘Timed Up and Go’ and either a 6-minute walk test or 10-m walk test as appropriate (performed by a specialist physiotherapist)
6. Medication utilization assessed using questionnaires before and after the intervention (performed by a nurse)
7. Adverse event log from starting hydrotherapy e.g. balance problems after each hydrotherapy session
8. Patient satisfaction assessed using a questionnaire at the end of the study

Overall study start date
15/06/2023

Completion date
30/10/2024

Eligibility

Key inclusion criteria
1. Adult MPS II patients (Hunter syndrome; all males aged 18-58 years), who suffer from joint pain and stiffness and/or post-surgical musculoskeletal pain, not responding to analgesic agents
2. Patients with reduced mobility and wheelchair reliance

3. Patients are able to give consent and feedback. In patients with limited capacity, their carers /parents may be asked to help complete the questionnaires.
4. Participants who pass the Trust hydrotherapy risk assessment at baseline

Participant type(s)
Patient

Age group
Adult

Lower age limit
16 Years

Upper age limit
58 Years

Sex
Male

Target number of participants
5

Key exclusion criteria
1. Contraindications to standard hydrotherapy: acute diarrhoea or vomiting, recent acute cerebral vascular accident (CVA)/deep venous thrombosis (DVT)/pulmonary embolism (PE), resting angina, resting shortness of breath, uncontrolled cardiac failure, weight in excess of evacuation equipment, proven chlorine allergy.
2. More severely incapacitated (severe learning needs) and/or immobile patients.
3. The objective Leeds Assessment of Neuropathic Symptoms and Signs (LANSS) score will be used to identify and exclude patients who suffer from predominantly neuropathic pain (rather than musculoskeletal nociceptive-driven pain). This assessment will be performed by a pain specialist.

Date of first enrolment
02/10/2023

Date of final enrolment
30/09/2024

Locations

Countries of recruitment
England

United Kingdom

Study participating centre
Salford Royal Organisation, Northern Care Alliance NHS Foundation Trust
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Sponsor information

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Sponsor type
Hospital/treatment centre

Funder(s)

Funder type
Charity

Funder Name
MPS Society UK

Results and Publications

Publication and dissemination plan
The results will be presented at the patients' meetings organised by the MPS Society, at MPS and LSD conferences and will be published in a manuscript

Intention to publish date
01/01/2025

Individual participant data (IPD) sharing plan
The datasets generated and/or analysed during the current study will be published as a supplement to the results publication

IPD sharing plan summary

Published as a supplement to the results publication