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The ACTIVE-FAB intervention to reduce sitting time and increase physical activity in adults with Fabry disease

Submission date
06/01/2025

Recruitment status
No longer recruiting

Registration date
09/01/2025

Overall study status
Completed

Last Edited
30/09/2025

Condition category
Nutritional, Metabolic, Endocrine

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

Plain English summary of protocol

Background and study aims
Fabry disease is an inherited metabolic disorder. Currently, treatments for Fabry disease are pharmacological and predominantly focus on the physical symptoms of the disease. In the general population and individuals with disabilities, reducing sedentary behaviour and increasing physical activity can be an effective, non-pharmacological treatment to improve mental health and quality of life. Such interventions have not yet been developed or evaluated in people with Fabry disease. A new sedentary behaviour and physical activity intervention has been designed with patients and healthcare professionals to support adults with Fabry disease to improve their physical health, mental health, and quality of life. The aim of this study is to test this new intervention, called ACTIVE-FAB, to see if it is acceptable and safe for adults with Fabry disease and what their experiences are when taking part in it.

Who can participate?
Adults with Fabry disease who are at least 18 years old, live in the United Kingdom, are able to walk without the help of another person, and are able to communicate in English.

What does the study involve?
Participants will be randomly allocated to the intervention group or the control group (continue with usual care and receive a general healthy lifestyle leaflet) for 3 months. Participants receiving the intervention will get (1) an initial consultation with a physiotherapist to discuss baseline sedentary behaviour and physical activity, and set goals, (2) two progress consultations with a physiotherapist to discuss engagement with the intervention, and review sedentary behaviour and physical activity goals, (3) an educational website, (4) a smartwatch that tracks sedentary behaviour and physical activity, and (5) an optional peer support group. All participants in the intervention and control groups will complete mental health and quality of life questionnaires. They will also have sedentary behaviour and physical activity measured using an activity monitor (called an activPAL) worn on the thigh for 8 days. They will complete these measures at baseline and 3 months after randomisation.
The researchers will assess if it is possible to deliver the intervention by seeing how long it takes to recruit enough participants to take part in the study, how many complete it and how many

provide information on each of the measures. The researchers will also talk to participants to see if they found the intervention and the information they are collecting are acceptable.

What are the possible benefits and risks of participating?
Participants may experience improvements in their health and quality of life by taking part in the study. The findings of the study will inform the design of a larger study in the future, which may lead to changes in healthcare for adults with Fabry disease to include an intervention like ACTIVE-FAB for managing the condition. By taking part, participants will be helping with this. Participants will also be able to keep the smartwatch after the study has ended.
Although a clinician will check it is ok for individuals to take part in the study, there is a small risk of experiencing some worsening of symptoms of Fabry disease. There is also a risk of injury while doing physical activity. There is a small risk of experiencing some distress during the focus group for those who take part in this.

Where is the study run from?
Brunel University London and the Royal Free NHS Foundation Trust (UK)

When is the study starting and how long is it expected to run for?
June 2024 to November 2025

Who is funding the study?
The Society for Mucopolysaccharide Diseases (MPS Society)

Who is the main contact?
Dr Daniel Bailey, daniel.bailey@brunel.ac.uk

Contact information

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Scientific, Principal investigator

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Additional identifiers

Clinical Trials Information System (CTIS)
Nil known

Integrated Research Application System (IRAS)
340412

ClinicalTrials.gov (NCT)
Nil known

Protocol serial number
Brunel University London ethical approval: 50235-NHS-Dec/2024- 53473-2, CPMS 65447

Study information

Scientific Title
The ACTIVE-FAB intervention to reduce sedentary behaviour and increase physical activity in adults with Fabry disease: a randomised controlled feasibility trial

Acronym
ACTIVE-FAB

Study objectives
This is a feasibility study to evaluate the feasibility, acceptability, and safety of conducting a randomised controlled trial of a sedentary behaviour and physical activity intervention (ACTIVE-FAB) for adults with Fabry disease.

Ethics approval required
Ethics approval required

Ethics approval(s)
approved 19/12/2024, Brunel University Research Ethics Committee (Brunel University London, Kingston Lane, Uxbridge, London, UB8 3PH, United Kingdom; +44 (0)1895266106; kate.dunbar@brunel.ac.uk), ref: 50235-NHS-Dec/2024- 53473-2

Study design
Mixed-methods randomized controlled feasibility trial

Primary study design
Interventional

Study type(s)
Other

Health condition(s) or problem(s) studied
Fabry disease

Interventions
Randomisation will be completed using an online randomisation tool, Research Randomizer (https://www.randomizer.org). An independent researcher will conduct the randomisation process. Participants will be individually randomised in a 2:1 (intervention: control) ratio using a fixed block size of three. The research team and the participant will be blinded to group allocation up until the point of group assignment.

Adults with Fabry disease will be recruited and randomly allocated to the intervention group or the control group (continue with usual care and receive a general healthy lifestyle leaflet) for 3 months. Participants receiving the intervention will get (1) an initial consultation with a physiotherapist to discuss baseline sedentary behaviour and physical activity, and set goals, (2) two progress consultations with a physiotherapist to discuss engagement with the intervention, and review sedentary behaviour and physical activity goals, (3) an educational website, (4) a smartwatch that tracks sedentary behaviour and physical activity, and (5) an optional peer support group. All participants in the intervention and control groups will complete mental health and quality of life questionnaires. They will also have sedentary behaviour and physical activity measured using an activity monitor (called an activPAL) worn on the thigh for 8 days. They will complete these measures at baseline and 3 months after randomisation.

Intervention Type
Behavioural

Primary outcome(s)
Feasibility, safety, and acceptability outcome measures calculated at the end of the study:
1. Participant eligibility will be calculated as: participants eligible / participants assessed for eligibility x 100
2. Recruitment rate will be calculated as: participants randomised / participants eligible x 100
3. Retention rate will be calculated as: participants who complete measures three months after intervention start / participants enrolled x 100
4. Completion rates for each outcome measure will be calculated as: complete data for the outcome measure / participants enrolled x 100
5. Trial safety will be assessed by calculating the frequency of pain crises, unscheduled hospital admissions, unscheduled GP appointments, any other adverse events and serious adverse events for the control and intervention groups
6. Acceptability of the intervention will be explored via focus groups with a subset of intervention participants

Key secondary outcome(s))

The secondary outcomes are preliminary estimates of intervention effects on:
1. Sedentary time measured using a physical activity monitor (activPAL4) over one full week
2. Physical activity measured using a physical activity monitor (activPAL4) over one full week
3. Anxiety measured using the Hospital Anxiety and Depression Scale (HADS)
4. Depression measured using the Hospital Anxiety and Depression Scale (HADS)
5. Quality of life measured using the EuroQol five-dimension questionnaire (EQ-5D-5L)
6. Goal attainment measured using Goal Attainment Scaling (GAS)

Sedentary time, physical activity, anxiety, depression, and quality of life will all be measured at baseline and 3 months after intervention start. Goal attainment will be measured 3 months after intervention start.

Completion date
30/11/2025

Eligibility

Key inclusion criteria
1. Consultant confirmed diagnosis of Fabry disease
2. Aged ≥18 years old
3. Have a Functional Ambulation Category rating of ≥3 (3 = ambulation with supervision, 4 = independent ambulation on level surfaces, and 5 = independent ambulation in all situations)
4. Lives in the United Kingdom

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
18 years

Upper age limit
100 years

Sex
All

Total final enrolment
45

Key exclusion criteria
1. Unable to provide informed consent
2. Unable to communicate in English to a sufficient level to permit engagement in the study
3. Taking part in another interventional study
4. Any medical conditions which result in the clinician deeming the individual ineligible to

participate (e.g. severe neuropathic pain, stage 4 heart failure or other condition resulting in breathlessness to a level that it could be unsafe to participate)

Date of first enrolment
20/01/2025

Date of final enrolment
02/08/2025

Locations

Countries of recruitment
United Kingdom

England

Study participating centre
Brunel University London
Kingston Lane
Uxbridge
United Kingdom
UB8 3PH

Study participating centre
Royal Free London NHS Foundation Trust
Royal Free Hospital
Pond Street
London
United Kingdom
NW3 2QG

Sponsor information

Organisation
Brunel University of London

ROR
https://ror.org/00dn4t376

Funder(s)

Funder type
Charity

Funder Name
The Society for Mucopolysaccharide Diseases (MPS Society)

Results and Publications

Individual participant data (IPD) sharing plan
The datasets generated and analysed during the current study will be stored in a publicly available repository (https://brunel.figshare.com/).

IPD sharing plan summary
Stored in publicly available repository

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Participant information sheet			07/01/2025	No	Yes
Participant information sheet	Participant information sheet	11/11/2025	11/11/2025	No	Yes