

Effects of exercise on Pompe disease

Submission date 30/09/2015	Recruitment status No longer recruiting	☐ Prospectively registered ☐ Protocol
Registration date 30/09/2015	Overall study status Completed	☐ Statistical analysis plan ☒ Results
Last Edited 15/04/2019	Condition category Genetic Diseases	☐ Individual participant data

Plain English summary of protocol

Plain English Summary under review.

Contact information

Type(s)

Scientific

Contact name

Mr Stephen Dando

Contact details

Charles Dent Metabolic Unit, Queen Square
London
United Kingdom
WC1N 3BG

Additional identifiers

Protocol serial number

19653

Study information

Scientific Title

A pilot study to investigate the effect of aerobic exercise training in adults with Pompe disease

Study objectives

The aim of this study is to investigate the effects of home based aerobic exercises on muscle and respiratory function, mobility and strength. The effects of cycling while receiving ERT will also be investigated.

Ethics approval required

Old ethics approval format

Ethics approval(s)

15/LO/0952

Primary study design

Interventional

Study design

Non-randomised; Interventional; Design type: Treatment

Study type(s)

Treatment

Health condition(s) or problem(s) studied

Topic: Metabolic and endocrine disorders, Musculoskeletal disorders; Subtopic: Metabolic and Endocrine (all Subtopics), Musculoskeletal (all Subtopics); Disease: Metabolic & Endocrine (not diabetes)

Interventions

Exercise, Cycling during Hospital based infusion. Participants will complete the initial, 3 month and final assessments and exercise testing at designated specialist centers in the UK.

Intervention Type

Other

Primary outcome(s)

Walking ability measured using a 6 minute walk test (6MWT) which will be recorded at baseline, 3 months and 6 months.

Key secondary outcome(s)

N/A

Completion date

16/09/2016

Eligibility

Key inclusion criteria

1. Aged between 16 and 70 years
2. Genetically proven diagnosis of late-onset Pompe disease
3. Attending Lysosome Centres in England
4. Can complete >50m in 6 minute walk test
5. Can transfer safely and independently on and off cycle ergometer
6. Can participate in all objective testing
7. Willing and able to attend specialist treatment centres
8. Willing to receive exercise testing and supervision in their own home

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Adult

Sex

All

Total final enrolment

11

Key exclusion criteria

1. Have been receiving Enzyme Replacement Therapy for less than 12 months
2. Able to walk more than 550m
3. Dependent on invasive ventilation during the day
4. Have cardiac issues for which exercise would not be recommended
5. Any current or previous orthopaedic medical condition for which exercise is contraindicated

Date of first enrolment

16/09/2015

Date of final enrolment

16/09/2016

Locations**Countries of recruitment**

United Kingdom

England

Study participating centre**Charles Dent Metabolic Unit**

University College London Hospital

Queen Square

London

United Kingdom

WC1N 3BG

Sponsor information**Organisation**

UCLH NHS Trust

ROR

<https://ror.org/042fqyp44>

Funder(s)

Funder type

Industry

Funder Name

Genzyme Therapeutics

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?
Abstract results	results presented at the Society for Inherited Metabolic Disorders meeting 2016	01/01/2017	15/04/2019	No	No
HRA research summary			28/06/2023	No	No