

# Effects of exercise on Pompe disease

<b>Submission date</b> 30/09/2015	<b>Recruitment status</b> No longer recruiting	<input type="checkbox"/> Prospectively registered <input type="checkbox"/> Protocol
<b>Registration date</b> 30/09/2015	<b>Overall study status</b> Completed	<input type="checkbox"/> Statistical analysis plan <input checked="" type="checkbox"/> Results
<b>Last Edited</b> 15/04/2019	<b>Condition category</b> Genetic Diseases	<input type="checkbox"/> 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

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers

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

**Study design**

Non-randomised; Interventional; Design type: Treatment

**Primary study design**

Interventional

**Secondary study design**

Randomised controlled trial

**Study setting(s)**

Other

**Study type(s)**

Treatment

**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**

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 measure**

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

**Secondary outcome measures**

N/A

**Overall study start date**

16/09/2015

**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

**Age group**

Adult

**Sex**

Both

**Target number of participants**

Planned Sample Size: 16; UK Sample Size: 16

**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**

England

United Kingdom

**Study participating centre**  
**Charles Dent Metabolic Unit**  
University College London Hospital  
Queen Square  
London  
United Kingdom  
WC1N 3BG

## Sponsor information

**Organisation**  
UCLH NHS Trust

**Sponsor details**  
University College London, Gower Street  
London  
England  
United Kingdom  
WC1E 6BT

**Sponsor type**  
Hospital/treatment centre

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

## Funder(s)

**Funder type**  
Industry

**Funder Name**  
Genzyme Therapeutics

## Results and Publications

**Publication and dissemination plan**  
Aim to publish the results in an appropriate peer-reviewed journal.

**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?
<a href="#">Abstract results</a>	results presented at the Society for Inherited Metabolic Disorders meeting 2016	01/01/2017	15/04/2019	No	No
<a href="#">HRA research summary</a>			28/06/2023	No	No