

Poster Abstract: Therapeutic

A National Pilot Study to Investigate the Effects of Sub-Maximal Aerobic Exercise in Adults with Late-Onset Pompe Disease

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BACKGROUND

Late-onset Pompe disease (LOPD) is characterised by progressive skeletal and respiratory muscle weakness. Sub-maximal (less than 70% of maximum capacity), aerobic exercise has improved walking and respiratory function in other neuromuscular diseases,¹ but little is known of the effects in Pompe disease.² An 18-month randomised controlled trial of enzyme replacement therapy (ERT) led to approval of alglucosidase alfa (Myozyme[®]) for the treatment of LOPD.³ In an effort to improve treatment outcomes, the potential benefit of adjunctive therapy, such as exercise, has previously been investigated. To date these studies have been small and uncontrolled, but results have been encouraging.^{4,5}

MATERIALS AND METHODS

The UK Pompe physiotherapy project will investigate the effects of sub-maximal aerobic exercise (SMAE) on muscle strength and function in patients with LOPD on Myozyme[®] over 6 months. Additionally, we propose that the increase in muscular blood flow that occurs through performing sub-maximal exercise will increase delivery of enzyme to skeletal muscle, when infused concurrently, and lead to an improvement in ERT efficacy.

Participants will be allocated to groups performing either SMAE not during infusion or SMAE during infusion (SMAE-I). All participants will use the Borg scale of exertion (Level 13) and heart rate monitors to ensure they are not exceeding sub-maximal effort (70% of maximal heart rate). Exercise programmes will be designed to be performed at a sub-maximal level for 30 minutes three times a week.

Participants in both groups will undergo supervised exercise testing on a cycle ergometer to establish an individual sub-maximal exercise programme to maintain heart rate (HR) between 60% and 70% HR max. Exercise programmes will be performed at home and all participants will be provided with exercise bikes and HR monitors. Those participants in the SMAE-I group will perform their designated exercise programme during fortnightly ERT infusions at home.

Exercise programmes will be supervised through monthly home visits by physiotherapists. Safety and adherence will be supervised further through weekly telephone calls and monitoring of exercise diaries. To reduce inter-observer variability inherent in muscle strength assessments, robust standardised assessment guidelines and techniques have been developed.

RESULTS

The results are currently pending. Primary outcome measure is the six minute walk test, and the secondary outcome measures are muscle strength and respiratory function as assessed by hand-held dynamometry, FEV1 and FVC. Measurements will be recorded at baseline, 3 months and at 6 months (study completion).

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CONCLUSIONS

Results of this study will hopefully inform the future physiotherapy and exercise management of patients with LOPD. It is the authors' belief that the combination of SMAE at the time of ERT infusion will have an increased positive effect on patient's physical capacity, muscular strength and lung function.

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