Progress Report

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Title of Project: Are alterations in energy expenditure responsible for obesity in Duchenne muscular dystrophy? A longitudinal exploration of energy requirements and body composition.

Summary: (approximately 1,000 words)
This longitudinal project aims to investigate the effect of disease progression on energy requirements and body composition in males with Duchenne muscular dystrophy (DMD); and to determine if a reduction in energy requirements is related to weight gain in these children. It has a translational focus in that the results will be used to inform the creation of specialised dietary protocols for weight management in DMD. Secondary aims of this project are to correlate changes in energy requirements and body composition with functional outcomes; and to investigate if accelerometry can be used as surrogate measure for energy expenditure in boys with DMD.

Approval from the Human Research Ethics Committee at Royal Children's Hospital was received early in 2015. This process exceeded expected timelines by approximately six months as we were required to obtain a legal agreement between all collaborating institutions. We have since opened for recruitment and have commenced baseline assessments. We aim to recruit 20 boys with DMD. We have identified 35 eligible participants and three boys have completed baseline assessments at the time of submission of this report. We have sent recruitment letters to the remaining families and we do not envisage issues with meeting our target sample over the next six months. Assessments are planned to be completed over three years so we will provide a final report to the Brain Foundation at this time.

Table 1 below summaries our project expenditure in relation to our submitted budget. We have purchased the doubly labelled water required for this project as per our submitted budget, and have secured a booking for analysis of our baseline urine samples at the end of 2015 at the University of Queensland. The funding received from the Brain Foundation has supported us to obtain additional funding internally from the Murdoch Childrens Research Institute to expand this project to other neuromuscular disorders including spinal muscular atrophy, congenital muscular dystrophy.

Table 1. Report on gift expenditure

Item	Details	Required	Cost per unit	$	Report on expenditure
Deuterium	0.05g/kg x 35kg x 20 subjects x 4 doses	140ml	$525/500mL	525	Purchased and received
18O	0.125g/kg x 35kg x 20 subjects x 4 doses	350ml	$2500/500mL	2,500	Purchased and received
DI-SIRMS analysis	11 samples x 20 subjects x 4 times	880 samples	$40/sample	35,200	Baseline samples (11 samples x 20 participants) have been booked for analysis
Spot urine containers	11 samples x 20 subjects x 4 times	880 samples	$0.50 each	440	Purchased and received
Shipping	$200 for stable isotopes shipping + $150 for sample transport to QLD x 4	-	-	800	Shipping cost for stable isotopes has been used.
TOTAL AMOUNT:				$39,465	

Hypothesis vs Findings

Our hypotheses remain as per our original submission.
In boys with DMD:
1) Total energy expenditure (TEE) will decline with disease progression.
2) There will be a relationship between TEE and body composition over time.
3) Declines in functional outcomes will be associated with changes in body composition.

Due to the longitudinal nature of this study we are unable to report on findings at this report. We will ensure that the Brain Foundation is kept up to date with our findings.

Unanswered Questions
To be determined.

What these research outcomes mean

To be determined.