

Can wearable sensor technology support a paradigm shift in paediatric rare disease research?

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INTRODUCTION

- ▶ Fifty percent of all rare disease present in childhood.
- ▶ Over 7 years only 17% of adult medicines authorised by European Medicines Agency completed paediatric trials. ^{1,2}
 - This is partly as these measures were developed for adults;
 - Partly due to the small participant pool.
- ▶ Many traditional endpoints such as the 6-Minute Walk Test (6MWT) cannot be used in all paediatric age subsets. ³
 - May not represent the patient experience.
- ▶ New Clinical Outcome Assessments (COAs) are needed to quantitatively assess how a patient feels, functions, or survives.
 - Can wearable sensor technology fill this gap?
- ▶ This feasibility study presents initial results and learnings regarding wearable sensor data as an outcome in paediatric rare disease research.

METHODS

- Two paediatric cohorts of rare disease participants
- ▶ Niemann-Pick C (NP-C) N=10
 - ▶ Duchenne Muscular Dystrophy (DMD) N=8
- A wrist-worn wearable device measured steps at home and feasibility was assessed qualitatively and quantitatively, with adherence to epochs defined as 8 epochs/day (4 hours).
- Quantitative experience recorded:**
1. Average daily steps,
 2. Epoch with most steps
 3. Average steps per 30-min epoch
- Qualitative experience recorded:**
1. Reasons for wearable non-adherence and replacement (lost / damaged / battery flat/ faulty)
 2. Clinicians and PI anecdotal accounts of challenges at the end of the study.

RESULTS

Demographics, 6MWT and wearable metrics from patients enrolled in the studies.

	NP-C	DMD
Demographics	n=10	n=10
Age (Median) in years	10.0	11.0
Age (range) in years	6-14	9-15
Sex M:F	5:5	8:0
6MWT Metrics	n=8	n=8
6MWT (Mean [SD])	444m (202m)	322m (120m)
6MWT (Median) in m	322m (120m)	325
6MWT Range in m	406m	178-522
ADM	1450.82	885.7
ADS	9582.71	4655.0
ASE	392.37	223.4

ADM = Average Daily Maximum: The maximum number of steps per epoch on each active day, averaged over all active days in the month.

2 ADS = Average Daily Steps: The total number of steps for a patient over a month divided by the number of active days.

3 ASE = Average Steps per Epoch: The total number of steps in a day divided by the number of active epochs. This daily average is then averaged over all active days in the month.

Specific issue identified from raw qualitative reports

"Some of our DMD boys didn't like the asymmetry. It is quite large on the wrist and the strap may need an shorter alternative for young children"

Specialist Physio

"She loves the colour purple - it's her favourite colour. But because of the soft texture of the strap she bites through it"

Mum of child with NP-C

	SPECIFIC ISSUE	PRO/CAREGIVER REPORT
NP-C	Disease severity - behavioural and advanced disease related non-ambulation Behaviour issues - biting through strap	Low engagement Loss of data; Required frequent replacement
DMD	Behavioural issues - removing watch due to asymmetric design	Loss of data;

DISCUSSION

- ▶ Collecting wearable data is feasible
 - Group differences arose which were not present in clinic
 - May be a sensitive addition to clinic measures
 - Relates to the patient and their environment
- ▶ Valuable insights into disease specific issues that should be used to develop future trials
- ▶ Could help combat small sample issues by adding additional insights into the data collected at clinic visits
- ▶ Further work is required to correlate the wearable device data with other clinical markers and self- or parent-report, however there is an increased momentum in the use of wearable sensors and apps as potential outcome measures in clinical trials

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