

WEARABLE TECHNOLOGIES FEASIBILITY AS AN OUTCOME MEASURE IN NIEMANN PICK-C

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Introduction

Niemann-Pick C (NP-C) is a rare inherited neurodegenerative autosomal recessive disorder primarily diagnosed in children. Disease symptoms include frequent falls, ataxia and behavioural problems. Doctors often assess NP-C impact using the Pineda Disability Severity Scale (PDSS)¹. Although effective in clinical assessment, the scale fails to enable patient or parental input. Therefore, patient reported outcomes (PROs) are important to understand disease impact at an individual level.

Wearable technologies allow daily continuous activity monitoring, and smartphone apps can facilitate PRO recordings and events in real-time. We report preliminary data and experience of an approach using this technology to understand NP-C disease activity. The study aim was to carry out a proof of concept evaluation to determine feasibility of using wearable technology to collect paired activity and PRO data in NP-C patients.

Methods

NP-C patients were recruited from 2 UK specialist centres. Doctors assessed each patient with PDSS¹, 6-Minute Walk Test (6MWT) and GAITRite.

Parents completed PROs about their child's disability; answering questions regarding Disease Impact severity, Family Impact and Wider Impact developed by the International Niemann Pick Disease Association and pushed at regular intervals to them via a mobile app. The Disease Impact severity PRO was developed from the PDSS domains, and matched accordingly.

Parents also self-reported any disease related event (e.g. choking episodes, falls, seizures.) on phone app in real-time. PROs and events were identified in collaboration with patient group.

Participants were also given a wearable device (a 3D accelerometer) which calculates step data in 30-minute epochs and transfers it via Bluetooth to a paired smartphone app downloaded to patient's personal (or carers) phone.

Funding

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Results (1)

Patient characteristics

- 10 patients aged 6-14 years old were recruited (male: female ratio 4:6).
- Mean PDSS at baseline was 11.1 (range 4-24);
- PRO Disease Impact of NP-C at baseline ranged from 2-18; mean: 10.
- NP-C Impact on Family ranged from 2-9 at baseline; mean: 5.6.
- NP-C Wider Impact ranged from 0-7; mean: 3.1.
- 6MWT scores ranged from 109m – 772m; mean 444m.
- Engaged patients (n = 7) with wearable device recorded between 1 - 235 days of data.
- Out of a total of 50 reported PROs, 18 were for Disease Impact and 16 each for both Family and Wider Impact. 51 clinical events were recorded by 7 patients.

Patient engagement with the wearable device:

3 patients recorded no active days of step data with the wearable device, 1 of these patients were non-ambulant but wanted to take part in the study anyway. 1 patient provided no PRO data via the phone app. 1 patient did not have 6MWT or PDSS completed.

From **Figure 1** and **Figure 2**, there is no statistically significant evidence of any correlation but this could be due to the low numbers of subjects when PDSS and 6MWT are compared with Active Days.

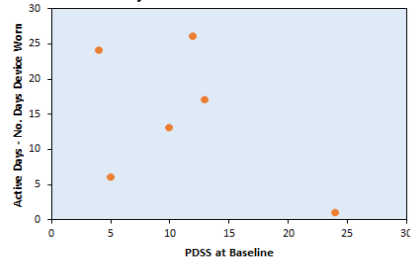


Figure 1: Wearable device activity correlated to PDSS.

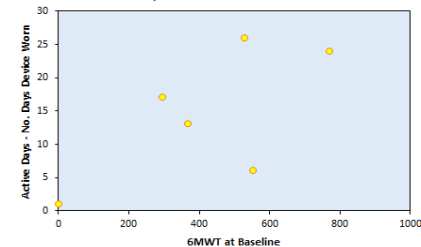


Figure 2: Wearable device activity correlated to 6MWT.

Results (2)

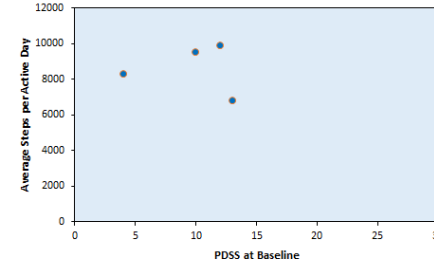


Figure 3: Average steps per active day of recording in relation to PDSS at baseline.

Early data observations

Detailed data from the pilot study is currently under analysis. With only 6 subjects with both Average Daily Steps per Active Day and baseline PDSS available, it is hard to establish the exact relationship to disease severity (PDSS), see **Figure 3**.

App: Events recorded

A total of 51 events were recorded collectively, 18 were for Other, 11 for Missed School, 8 for Fall, 7 for Diarrhoea, 4 for Seizure and 3 for Choking (**Figure 4**).

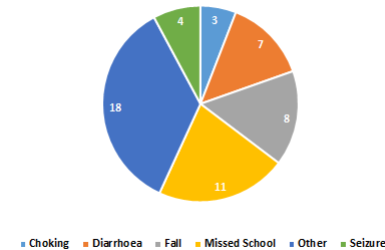


Figure 4: All event types recorded for patients collectively.

Organisational and technical challenges experienced

Some technical issues were experienced with the app and wearable device:

- Difficulties syncing the device to mobile phone.
- Bluetooth failures
- Battery failures
- Lost and chewed devices

Discussion & Conclusions

We have demonstrated with this pilot study that it is feasible to use wearable accelerometer devices as a tool for measuring disease activity. Full analysis of the comprehensive data collected is under way and will be reported in the coming months.

Initial analysis from Doctor assessed PDSS and Parent reported Disease Impact PRO indicates that Doctor scored higher disease severity / impact than parents.

Future analysis will aim to identify how well the wearable device data correlates with other measures of disease severity, e.g. 6MWT, GAITRite assessment and clinical evaluation by clinicians.

Furthermore, for the patients that had long follow up data analysis will be done to see how this changes over time.

The study provided an insight to organizational and technical challenges of deploying such technology. Using technology outside of the hospital setting has a host of challenges both technically and logistically and an accessible and approachable research team is integral to such a projects success.

Data collected allows for comparison between objective clinical measures of disease severity assessed in clinic, with real-time PROs completed remotely. Further feasibility work is required to establish patient engagement with wearable technologies.

Full comprehensive data analysis collected is under way. Analysis will aim to identify how well wearable device data correlates with other measures, e.g. 6MWT and GAITRite assessment.

References

- 1) Itturiaga C, Pineda M, Fernández-Valero EM, et al. Niemann-Pick C disease in Spain: Clinical spectrum and development of a disability scale. *J Neurol Sci* 2006;249:106.