Aparito's six year journey in lysosomal storage disorders

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INTRODUCTION

Lysosomal storage disorders (LSDs) are characterised by their vast heterogeneity[1]. Additionally, as a group of rare diseases there may still be pathophysiological mechanisms which need to be understood further[2].

As such, diagnosing or administering the necessary treatments for patients can be difficult.

Remote medical technology (med tech) tailored for LSDs in the form of a smartphone app, paired with a wearable device can aid drug development or clinical activities through:

- ***Continuous data aggregation*** *- detailed tracking of disease progression - enhancing understanding of disease pathophysiology and therapeutic intervention*
- ***Extra-clinical oversight*** *- detailed tracking of disease progression - enhancing understanding of disease pathophysiology and effect of therapeutic intervention*
- ***Electronic clinical outcome assessments (eCOA)*** *- understanding the patients perspective to identify additional needs*

METHODS

The med-tech developed for LSDs consists of:

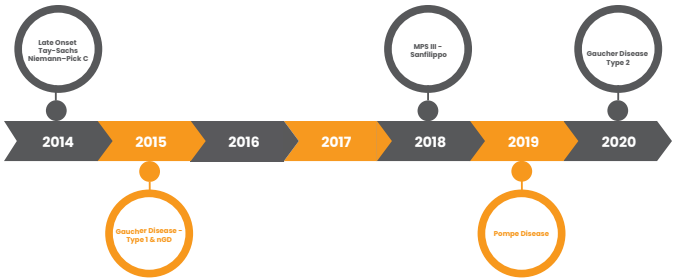
- *A mobile app for completion of eCOA, daily diary and video capture*
- *Clinician dashboard for real-time patient specific and cohort wide assessment*
- *A wearable device integration for capturing physiological parameters*

The longitudinal aggregation of psychosocial and physiological data provides a well rounded view of the patients' experience.

A med-tech solution to collect patient data

Collecting data from a device

Aparito's six year journey in LSDs



RESULTS

Over a six year period, med-tech provisions have been designed for 7 LSDs, with the primary focus across studies differing:

- ***Pilot study:*** *ushered in the initial med-tech iteration*
- ***Proof of concept:*** *developing proof of concept for med-tech application in LSD*
- ***Natural history study:*** *charting disease progression over time*
- ***Regulated pivotal studies:*** *utilising wearables, videos, PROs and more to monitor the safety and efficacy of Investigational Medicinal products (IMPs)*

The patients involved in studies completed between … were varied, with ages ranging from 4-64 years.

CONCLUSION

Remote med-tech has successfully been deployed in various LSD studies over the past six years with the following being observed:

- *Generation of informative longitudinal data sets across LSDs*
- *Acceptable adherence rates showing its accessibility to patients*

With the benefits of remote med-tech for patients and clinicians alike being apparent, and with more med-tech based studies on the horizon for LSDs, there is technological momentum at hand from which LSD patients can benefit from.

REFERENCES

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