

# Empowering the GM1 and GM2 community with a co-created education resource about a natural history study

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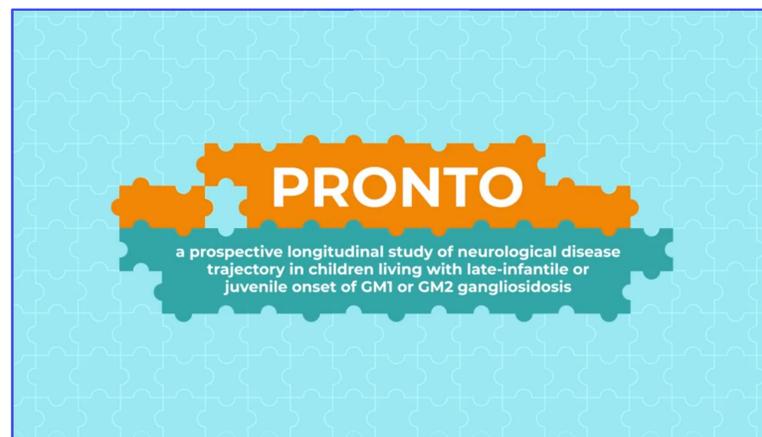
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## RATIONALE

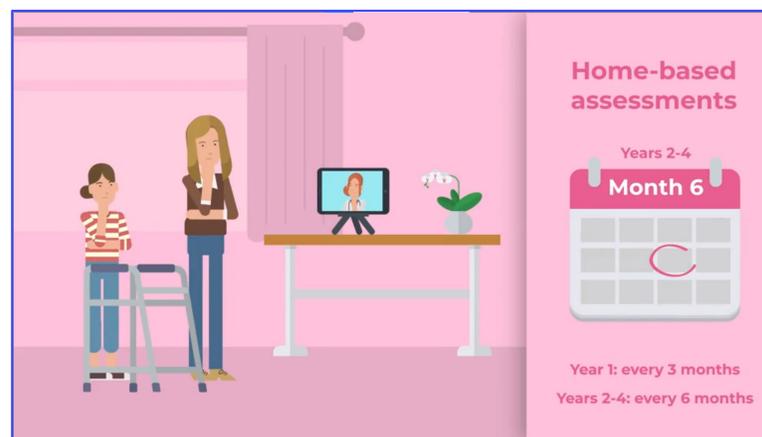
There are difficulties in recruiting and retaining participants in natural history studies in any therapy area. These include a lack of participant understanding of clear benefits and, often, the long-term commitment required<sup>1</sup>. This is exacerbated in rare disease natural history studies by a plethora of well-documented and multi-faceted additional challenges, including a potentially small and globally-dispersed patient population to initially recruit from, challenges accessing appropriate support networks, and increased caregiver burden<sup>2</sup>. It is evident that these challenges are a barrier to recruitment and retention, often delaying clinical trials and increasing study costs<sup>3</sup>. Considering these challenges, this project presents a novel way to potentially enhance participant knowledge and subsequent motivation to participate in a natural history study investigating GM1 and GM2 gangliosidoses.



Figures 1 & 2. Extracts of the co-created, animated, video content describing what participation in the PRONTO study entails

## PROCESS

Azafaros launched the PROspective Neurological Disease TrajectOry (PRONTO) natural history study investigating GM1 and GM2 gangliosidoses in late 2022. Working with Cognitant, specialists in creating educational resources for patients and caregivers, they engaged in a co-creation process to create patient-led digital education resources about their upcoming study. By convening key stakeholders, including KOLs, clinicians, advocacy groups, patients, and caregivers, from Europe and the US, Azafaros created a unique multi-lingual digital knowledge centre that utilises different digital media to detail what a natural history study is, what participation in PRONTO will entail, and how the study will be assessed. As the brain processes visual information 60,000 times faster than text, presenting information in video format promotes accessibility<sup>4</sup>. By hosting this content in a digital knowledge centre, clinicians, advocacy groups and families can send this content directly to participants and caregivers, enabling them to view and share the digital content away from the clinic and in their own time.



## ENGAGEMENT

The impact of this digital intervention is assessed through patient feedback and engagement metrics. In the first three months...



250 individual users accessed the platform



... from 13 different countries



... with an average engagement time of 7 minutes 30 seconds

Whilst trial engagement will be continually assessed, this clearly demonstrates an appetite for curated, effective, and understandable information from patient communities.

## IMPLICATIONS

These initial findings demonstrate that:

- There is a clear appetite for patient-centred, and co-created, content informing families about what clinical trials entail
- Families are happy, and excited, about being involved in the co-creation process, and provide valuable insights to help validate educational needs and learning preferences
- Investing in co-created, interactive, and engaging information materials has the potential to minimise study attrition and demonstrate long-term cost-effectiveness

## REFERENCES

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