Utilising co-created digital health information in rare paediatric neurological disease clinical studies

Louis S Stokes[1], Daniel Lewi[2]

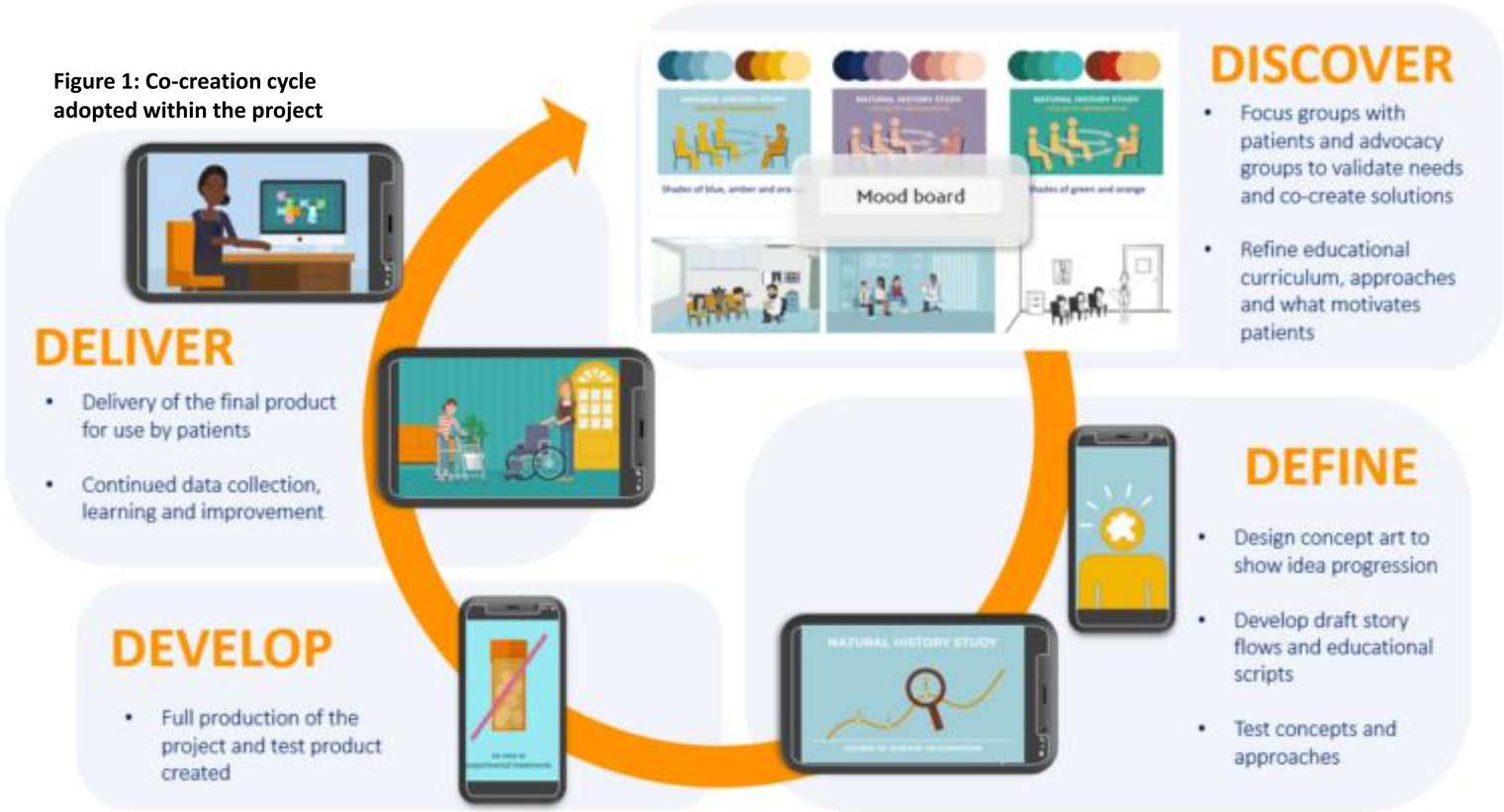
Introduction

- Recruiting participants to rare disease natural history studies is inherently challenging due to the small patient population to recruit from.
- This challenge is often further compounded as motivation to engage in paediatric trials is reduced by the lack of clear and understandable information materials shared with caregivers about what will be expected from them, and their child, throughout participation.
- PRONTO, a natural history study, designed by Azafaros to investigate GM1 and GM2 gangliosidoses, launched in 2022 and took an innovative approach to overcome the aforementioned challenges

Methods

- We undertook an iterative co-creation process (see Figure 1) that ensured a user-centred design resulting in a patient-led digital education resource informing participants about their upcoming study.
- By convening key stakeholders, including KOLs, clinicians, advocacy groups, patients, and caregivers, a unique multi-lingual digital knowledge centre was created that utilises 2D animation, amongst various other digital media, to detail what a natural history study is, what participation in PRONTO will entail and how the study will be assessed.
- Digital content can be forwarded to prospective participants by clinicians and researchers

Figure 1: Co-creation cycle adopted within the project



Results

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.

[1]Cognitant, Oxford, UK, [2] The Cure & Action for Tay Sachs Foundation (CATS)
Contact email: louis.stokes@cognitant.com