Development of a digital app and international real-world observational study in Charcot-Marie-Tooth disease

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BACKGROUND
Charcot-Marie-Tooth disease (CMT) is a progressive hereditary motor and sensory neuropathy that affects the peripheral nervous system, leading to muscle atrophy in the limbs and impaired sensitivity to touch, vibration, heat, and pain. CMT compromises patient and carer lifestyle, everyday activities, and career and family choices. CMT is rare, and there has been little research into its impact on patients’ and carers’ lives. The collection of real-world data, direct from patients, may therefore provide valuable insights. Real-world data collection promotes greater and broader patient representation than is typical in clinical trials. It permits a fuller assessment of the effects of disease and treatment, by removing the constraints enforced by the trial setting. Digital technology has the potential to transform real-world data collection, but its use is still relatively novel. It therefore seems important to develop best practices for digital real-world data collection.

METHODS
Observational study overview
The study, under analysis, is an ongoing observational study to evaluate the impact of CMT and its treatments on the quality of life of a large and diverse participant population, as the time required for onboarding individual sites is eliminated. The study is being implemented according to best practice: including industry-standard data storage and privacy for all users. The study does not present any identifiable risk to participants. A Scientific Advisory Board (SAB) has been convened, comprising industry experts, patient advocates, clinically experienced clinicians, and researchers.

Recruitment strategy
Recruitment and communication with study participants is being undertaken via social media, email, PAO websites, physical distribution of materials (e.g. postcards in doctors’ surgeries; Figure 1 and word of mouth). Participants are being recruited from each of the scope countries (France, Germany, Italy, Spain, the US, and the UK), and international CMT experts, for use in an observational study to understand the impact of CMT on patient outcomes. A key secondary objective was to analyse recruitment rates for the observational study to date.

RESULTS
Recruitment rates and participation by state (US, Day 1–23)
Recruitment has been rapid, with 192 participants enrolled in the first 23 days of the study. In addition, a broad age range and spread of materials (e.g. postcards in doctors’ surgeries; Figures 2 and 3, respectively). Recruitment was rapid, with 192 participants enrolled in the first 23 days of the study, in addition, a broad age range and number of states are represented in the study population.

Figure 5: Cumulative recruitment per US state, Day 1–23

Figure 6: Participants enrolled by state (US), Day 1–23

Figure 7: Top 10 states by recruitment, Day 1–23

CONCLUSIONS
This analysis has presented the successful creation of a digital app study in collaboration with patients and PAOs, for use in an observational study of the real-world impact of CMT. The use of digital technology and the collaborative development approach have been key to the successful recruitment and engagement of study participants to date. The study presentations provide a powerful illustration to other digital real-world observational studies. In the ongoing observational study, we will generate valuable real-world data on the impact of CMT, which will help improve patient care.