Development of a digital app and innovative recruitment for an 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 lifestyles, 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 is therefore important to develop best practices for digital real-world data collection.

Study objectives

The primary objective was to evaluate the development of a digital app, co-created with patients, Patient Advocacy Organizations (PAOs), and international CMT experts, for use in an observational study to understand the impact of CMT on patients' daily lives.

A key secondary objective was to analyze recruitment rates for the observational study to date.

METHODS

Observational study overview

The study under analysis is an ongoing observational study to evaluate the impact of CMT and its treatment on patients (burden of disease, natural history and treatment, and medical, social and pharmacoeconomic effects).

The study has been co-developed by the healthcare research company, Vitaccess, the biotech company, Pharnext, CMT PAOs and international CMT experts.

Inclusion criteria are as follows:

- Aged ≥18 years
- Diagnosed with any type of CMT
- Resident in a scope country (France, Germany, Italy, Spain, the UK, and the US)
- Willing to use, and capable of using, own smartphone.

There are no specific exclusion criteria.

Planned enrollment is ≥2,000. The maximum number of participants is 10,000. Recruitment is ongoing.

Participants will be followed up for two years.

Development of study app

The observational study utilizes the MyRealWorld™ digital platform, developed by Vitaccess.

- Participants download a 'bring your own device' study app onto their phone, within which they can register for the study, provide informed consent, and complete study surveys.
- A user portal allows researchers to explore aggregated, anonymized data in near real-time, via simple, highly visual dashboards; data can be filtered and analyzed according to pre-set criteria (e.g. disease subtype, age, gender).
- The platform is user-friendly, with a low data entry and collection burden.

A bespoke app was developed for the study.

- Patients and PAOs were consulted on the proposed design, including branding and color scheme, language and tone, navigation, and potential additional functions.
- The app underwent pre-launch testing with patient volunteers.

Data management and scientific rigour

The study is being implemented according to best practice:

- A Scientific Advisory Board (SAB) has been convened, comprising PAO representatives and clinicians, and representatives from Pharnext and Vitaccess.
- Ethical approval has been obtained.
- Participants provide informed consent prior to enrolling, and are free to withdraw at any time.
- The study does not present any identifiable risk to participants.
- The study app has been adapted for each study country.
- Best practice technologies and security policies ensure industry-standard data storage and privacy for all users.

Data capture

The data acquisition plan has been developed collaboratively by all stakeholders.

Participant background data are collected upon study registration.

Participants complete a monthly study survey, which takes approximately 21 minutes, and includes the following:

- EQ-5D-5L (a generic health-related quality of life [HRQoL] instrument)
- The Work Limitations Questionnaire
- 'Patient-Reported Outcomes (PRO) instruments measuring specific symptoms (pain intensity and interference, fatigue and cramps)
- Sleep quality instrument.

Also, instruments measuring function (fear of falls, lower limb function, upper limb function) are completed every three months; one instrument is completed each month in turn, taking approximately 3–7 minutes each.

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 in a staggered sequence: first the US, followed by the UK, Italy, Germany, Spain, and France.

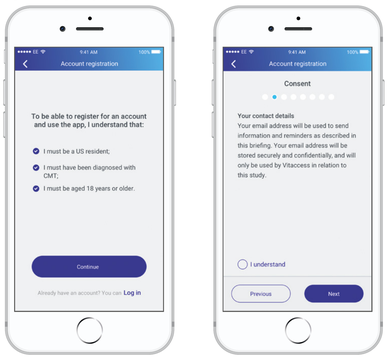
Potential participants are invited to the study via an email from their local PAO, containing a link to the app.

Further recruitment is being performed via social media and word of mouth, with potential participants able to download the study app from an app store.

To register, participants install the app, complete an eligibility questionnaire, review participant briefing materials, and complete informed consent questions (Figure 2).

Figure 1: Sample from recruitment postcard

Figure 2: Eligibility questionnaire and informed consent (for US participant)



RESULTS

CMT&Me study app

Collaboration with patients and PAOs has culminated in a study app called CMT&Me.

Study participants receive email and in-app notifications to prompt them to complete surveys.

In addition to generating data, the app gives participants access to a range of condition management features, which help them to understand and manage their disease, and are designed to promote engagement and persistence (Figure 3).

Participants can also record their symptoms in a diary (Figure 4).

Figure 3: Condition management features

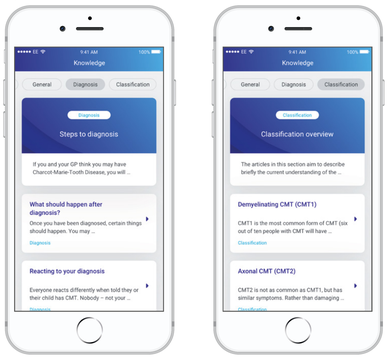
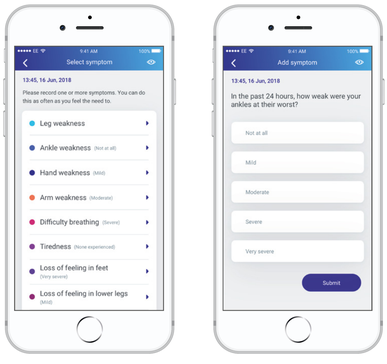


Figure 4: Symptoms diary



Recruitment rates

Recruitment data are currently available for the first country, the US, up to Day 23 of enrollment. Recruitment rates by day, US state, and age are presented in Figures 5, 6, and 7, respectively.

Recruitment has been 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 curve: US, Day 1–23

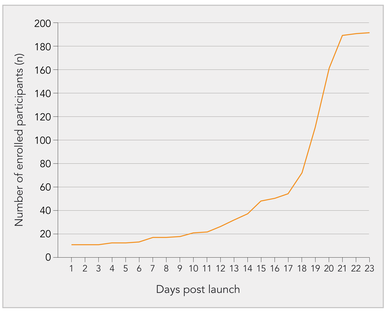


Figure 6: Enrolled participants by state: US, Day 1–23

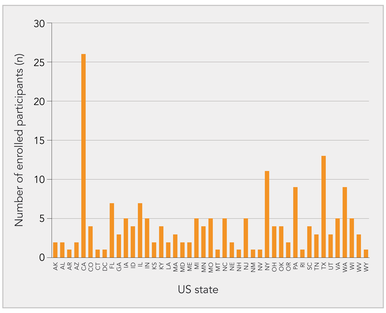
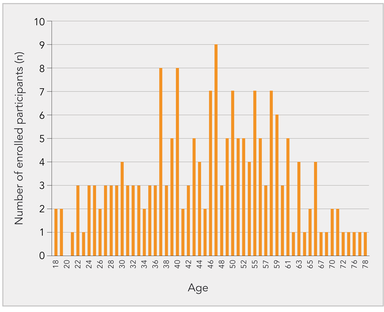


Figure 7: Enrolled participants by age: US, Day 1–23



Discussion

Data collected so far indicate that the study will efficiently recruit a large and diverse participant population, providing strong representation of different subgroups.

The success of recruitment is likely to be attributable to the digital approach, and the collaborative development of the recruitment strategy and study app:

- Relative to a physical study, digital studies can immediately recruit across a wider geographical area, and from a larger population, as the time required for onboarding individual sites is eliminated.
- Digitization of registration means that study participants are able to sign up at a time and location convenient to them, thus reducing participation burden.
- Collaboration with patients and PAOs fosters a sense of openness and communication between participants and investigators, and encourages the word-of-mouth spread of information about the study.
- The co-development approach also ensures that enrollment materials are user-friendly and engaging, thus making the registration process straightforward.

CONCLUSIONS

This analysis has presented the successful creation of a digital study app 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 presented here provides a powerful blueprint for other digital real-world observational studies.

It is hoped that the ongoing observational study will generate valuable real-world data on the impact of CMT, which will help improve patient care.

ISPOR Europe 2018, Barcelona, Spain, 2018. PND148