Innovative partnerships with patients and patient advocacy groups in digital observational rare disease studies

Llewellyn S, Bagshaw E, Williams H, Larkin M - Vitaccess Ltd, Oxford, UK

Objectives

To investigate the effect of co-creating digital study apps with all stakeholders for use by rare disease patients in observational real-world studies.

Methods

Custom-made "bring your own device" (BYOD) iOS and Android mobile apps (see Figure 1) were developed for use in short- and long-term observational studies to evaluate the impact of rare diseases (namely Charcot-Marie-Tooth disease [CMT] and transfusion-dependent β-thalassemia [TDT]) in the real-world.

Figure 1: BYOD mobile apps included in this evaluation

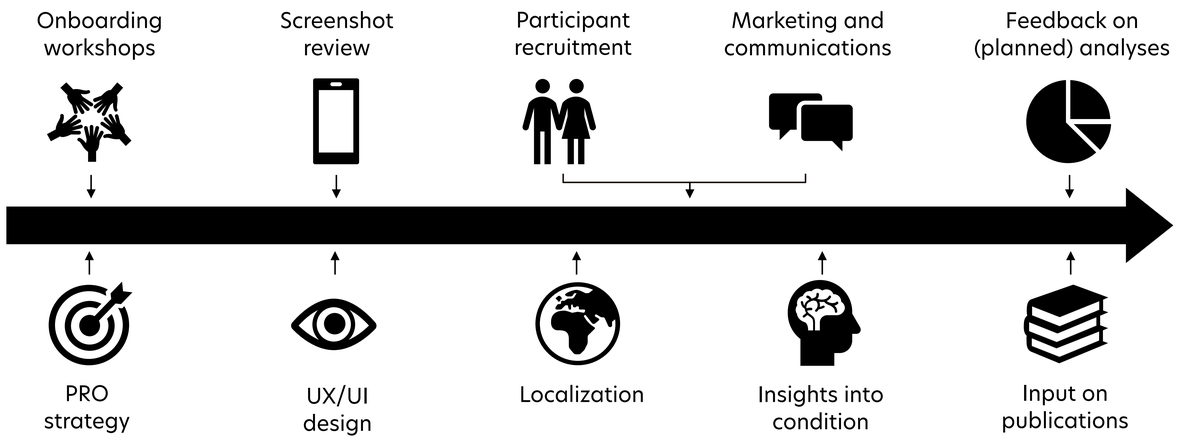
myThalLog

CMT&Me

In line with published recommendations[1], creation and ongoing development of the apps was informed by input from potential participants and patient advocacy groups (PAGs) [see Figure 2].

This included one-day onboarding workshops with PAG representatives and patients from France, Germany, Italy, Spain, the United Kingdom, and the United States. Further pilot testing of the study apps with designated patient "super users" was conducted to obtain feedback on the data acquisition strategy (e.g., review of patient-reported outcome [PRO] instruments for patient relevance, domains to be added, others to be deprioritized).

Figure 2: Areas of the digital observational studies where patient and PAG input was incorporated



To improve the value of the apps to participants and promote ongoing engagement, feedback from users is being sought as the studies progress. Feedback of this type is collected through a variety of interactive means (e.g., online polls, focus groups).

Results

Initial patient and PAG feedback provides important insights into the tailoring of the apps, including:

- the components of burden of disease that need to be assessed for a full evaluation (example provided in List 1);
- the app user experience;
- the relevancy of custom-made survey questions/ PRO instruments and acceptable frequency of their administration;
  For example, in the "CMT&Me" app, the list of orthotics/ walking aids that users could pick from was adjusted during app localization based on information from PAG representatives on the in-country availability of different orthotics/walking aids.

List 1: Insights from patients and PAGs on the burden of CMT

Symptoms
Voluntary muscle weakness in the eyes, face, proximal limbs, throat, and neck. Pain. Difficulty breathing. Fatigue. Headache. Bladder/bowel function.

Compromised physical function
Lower extremity. Upper extremity.

Activities of daily living
Changes to day-to-day living. Forced to slow down.

Psychological function
Anxiety. Stress. Depression. Feels like a burden to others.

Social function
Relationships with others.

Work or School
Missed school. Missed work due to sickness. Left work altogether. Changed occupation.

Financial burden

Impact to health-related quality of life
Physical, mental, social and emotional well-being.

Ongoing feedback is useful to understand how participants interact with the apps, any challenges they may face when using them, and any additional features they may find useful in helping to manage their conditions.

Discussion and Conclusions

Patients and PAG representatives can provide invaluable input throughout the duration of digital observational rare disease studies, thereby ensuring that the study apps capture all relevant data and provide the optimum user experience.

This innovative way of working has been put into practice for ongoing studies in rare diseases such as CMT and TDT, but also melanoma (as described in Au *et al.*, 2018[2]).

It will also be adopted for upcoming digital observational studies in cystic fibrosis and myasthenia gravis, which will receive input from patients and PAGs in Belgium, Canada and Ireland, in addition to those mentioned previously.

References

[1]Berger *et al. Pharmacoepidemiol Drug Saf.* 2017; 26(9): 1033-9
[2]Au *et al. Value Health.* 2018; 21: S38

vitaccess