2nd European CMT Specialists Conference
Antwerp, 23-25 October 2025

Poster P29

Better Balance-CMT: Protocol for a randomised, controlled, efficacy and implementation trial of a home-based, balance training intervention for people with Charcot-Marie-Tooth Disease

G. Ramdharry (1,2), L. Lee (1,2), M. Dudziec (2), M. Reilly (1,2)

(1) Queen Square Centre for Neuromuscular Diseases, National Hospital for Neurology and Neurosurgery, University College London Hospitals NHS Foundation Trust, London, UK
(2) Department of Neuromuscular Diseases, UCL Queen Square Institute of Neurology, London, UK

Multi-sensory rehabilitation has shown promising effects in people with sensory loss, and resistance training can improve proximal lower limb muscle strength. Studies of multi-sensory rehabilitation with and resistance training can improve balance in people with CMT in specialist clinics. We developed a pragmatic, home-based balance rehabilitation program and a proof-of-concept study found it to be safe and acceptable for people with CMT with excellent engagement. This will now be tested at scale through the Better Balance-CMT (BB-CMT) trial.

Methods: We will partner with patients and stakeholder to co-produce web-based resources for the BB-CMT intervention. It will be delivered at home by trained physiotherapists, through 3 face-to-face sessions, using self-management principles, digital materials and remote support.

We will then conduct a randomised, single blinded, two arm trial of the BB-CMT intervention compared to treatment as usual. A hybrid-1 trial design is planned, with the primary aim of exploring efficacy of BB-CMT and the secondary aim of exploring potential barriers and facilitators to “real-world” implementation into practice. The program will last 12 weeks and compared to a 12-week control period. A 12-week open label is included to assess continued engagement and carry over.

The primary outcome measure is the Berg Balance Scale (BBS), calculating mean difference in BBS score between the BB-CMT and the control group using a linear regression, adjusted for baseline BBS score. A target sample of 84 participants is based previous studies of balance training in CMT, with a detectable standardised effect size of 0.66, at 80% power and 5% 1-sided alpha, allowing for a 10% drop out. Participants will be recruited from 6 NHS hospitals in England.

Conclusion: Funding for this work has been acquired through a National Institute for Health Research award. The co-production work will start in autumn 2025, with the BB-CMT trial to commence in 2026.