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# Presentation PL3-05

## Therapeutic Potential and Safety of allogeneic mesenchymal stromal cell (EN001) in CMT1A and CMT1E: First-in-Human Evidence from South Korea

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Charcot–Marie–Tooth disease (CMT) is the most common inherited peripheral neuropathy, affecting 1 in 2,500 individuals, with an estimated 300,000 patients in Europe. Over 140 causative gene variants have been identified, with duplication of PMP22 responsible for nearly 50 % of all cases. Despite being first described in 1886, no EMA-approved therapy for CMT exists. Regenerative medicine offers a novel approach for intractable neuromuscular diseases such as CMT. Mesenchymal stromal cells (MSCs) exert regenerative and immunomodulatory effects via homing and paracrine actions. MSCs can be administered allogeneically due to their immune-privileged nature, allowing for off-the-shelf use. MSC-derived factors support immune-modulation, anti-apoptosis of nerves and muscles, and remyelination. In our previous preclinical studies, Wharton's jelly-derived MSCs (WJ-MSCs) enhanced Schwann-cell proliferation and remyelination in C3, C22, and Tr-J CMT mouse models. EN001, an allogeneic WJ-MSC therapy developed by ENCell Corp., was evaluated in two single-dose first-in-human studies in South Korea: one in patients with CMT1A (NCT05333406) and another in patients with CMT1E (NCT06218134). Safety and tolerability were the primary endpoints; exploratory efficacy was assessed via the CMT Neuropathy Score version 2 (CMTNSv2), Functional Disability Scale, Overall Neuropathy Limitation Scale (ONLS), and nerve conduction studies (NCS), etc. EN001 was well tolerated, with no dose-limiting toxicities and serious adverse events. Only transient, mild adverse events unrelated to EN001 were observed. Both patients with CMT1A and CMT1E showed reductions in CMTNSv2 after a single administration of EN001, indicating clinical improvement. These findings demonstrate the safety and preliminary therapeutic potential of EN001 and suggest it could become a viable therapeutic option for CMT. Large, multi-ethnic randomized controlled trials are now required to confirm safety and efficacy of EN001.