

# Pre-clinical development of AAV-CRISPR-based PMP22 normalization gene therapy for Charcot-Marie-Tooth 1A



NEUROSCIENCE	Non-Clinical
Product Type	Adeno-associated virus (AAV)
Indication	Charcot-Marie-Tooth 1A (CMT1A)
Target	Peripheral myelin protein 22 (PMP22)
MoA(Mechanism of Action)	Normalization of PMP22 expression level in schwann cell by editing of regulatory region of PMP22 gene by CRSIPR/Cas9
Competitiveness	First-in-class product directly targeting PMP22 expression level which is the fundamental cause of CMT1A. Normalization (not knockdown) of PMP22 expression specifically in schwann cell
Development Stage	Non-Clinical
Route of Administration	Intrathecal or intravenous