

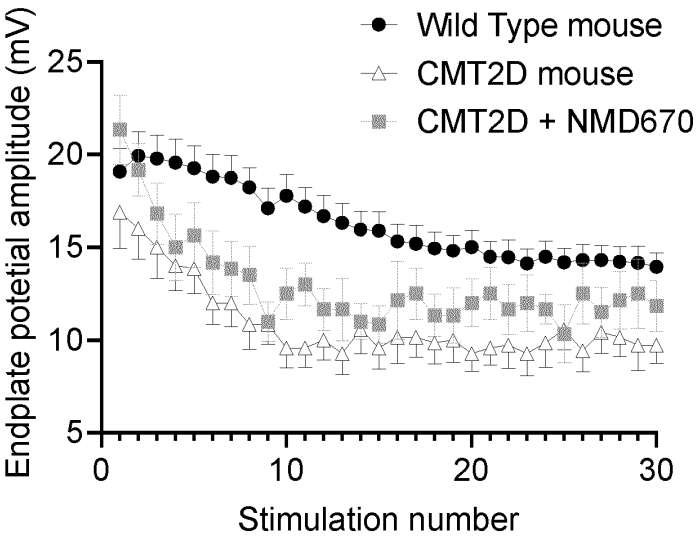


Fig. 1

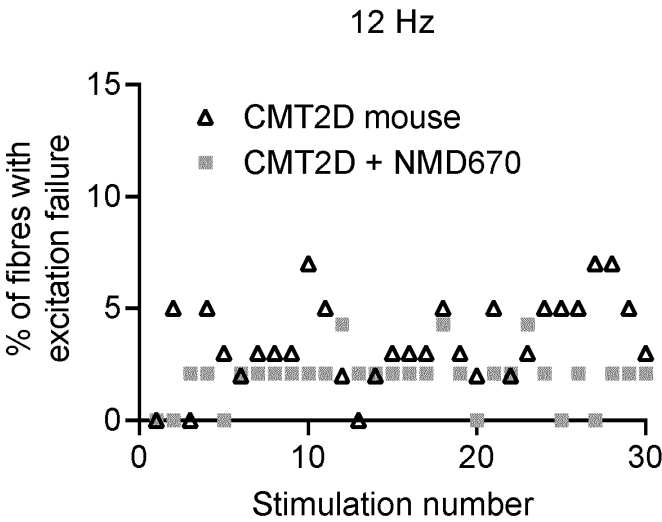


Fig. 2A

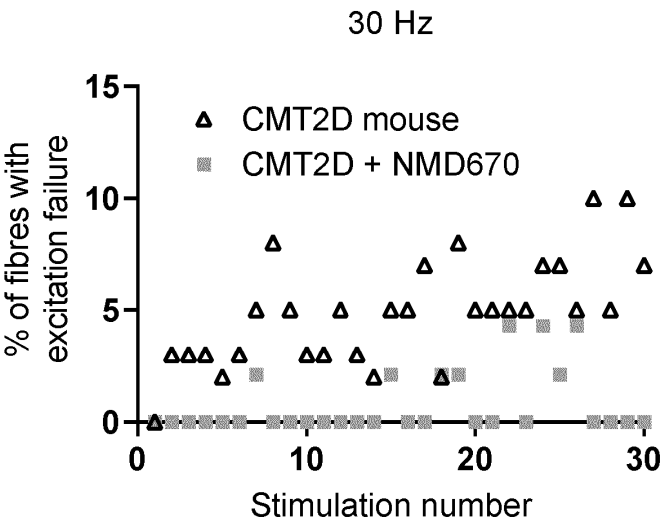


Fig. 2B

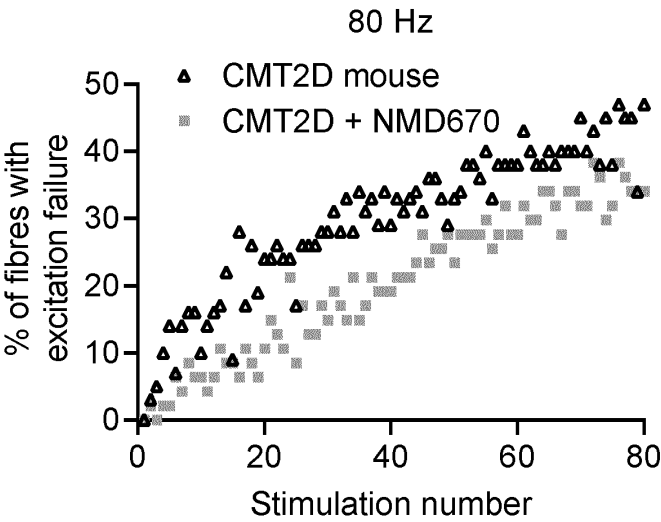


Fig. 2C

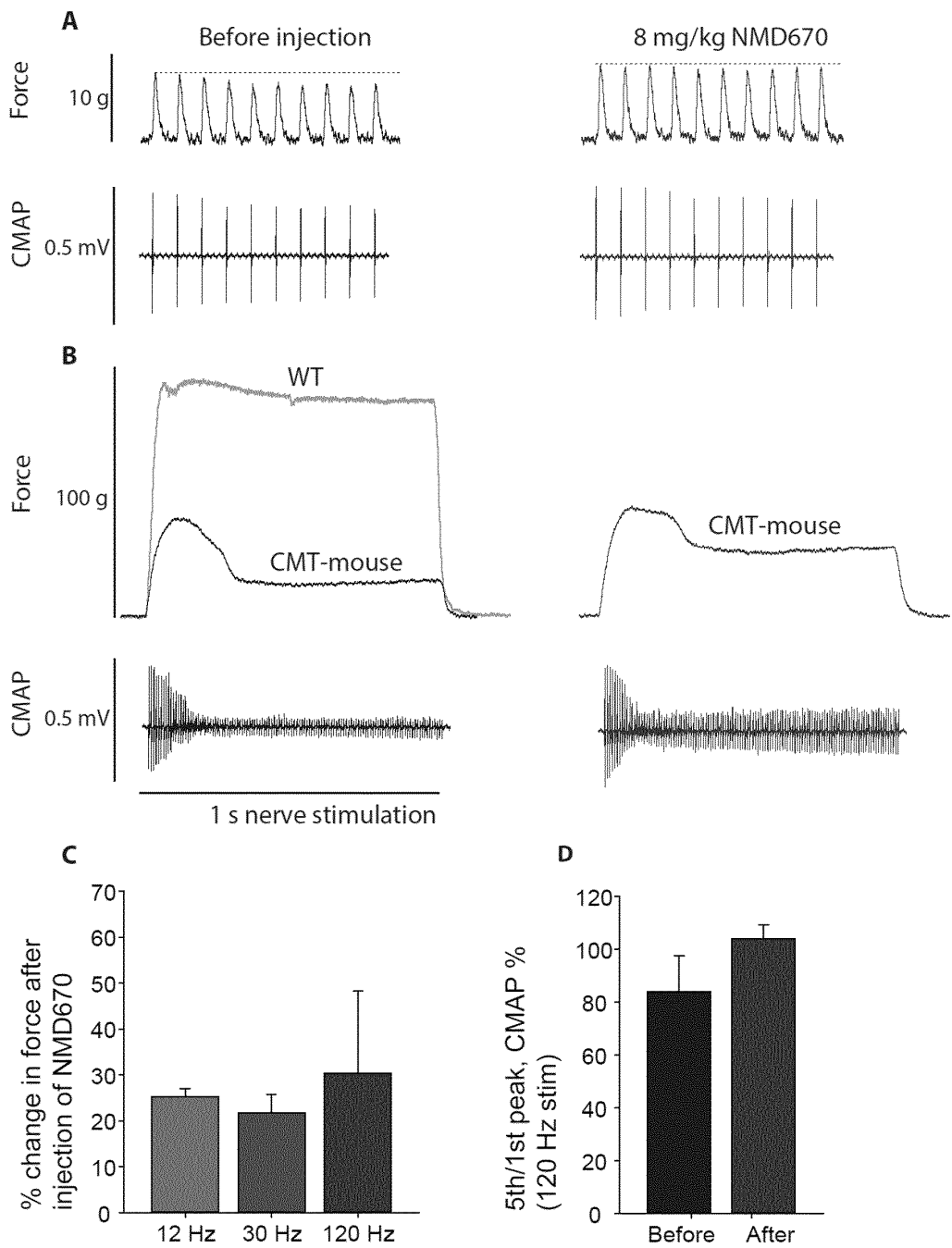


Fig. 3

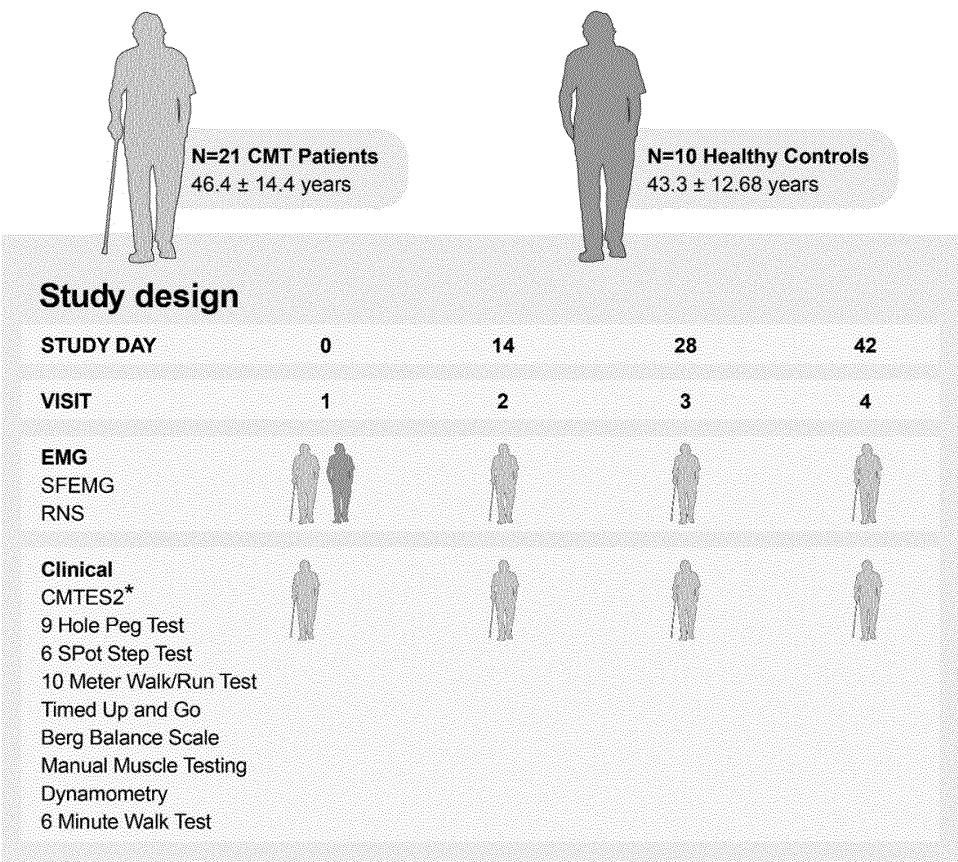


Fig. 4

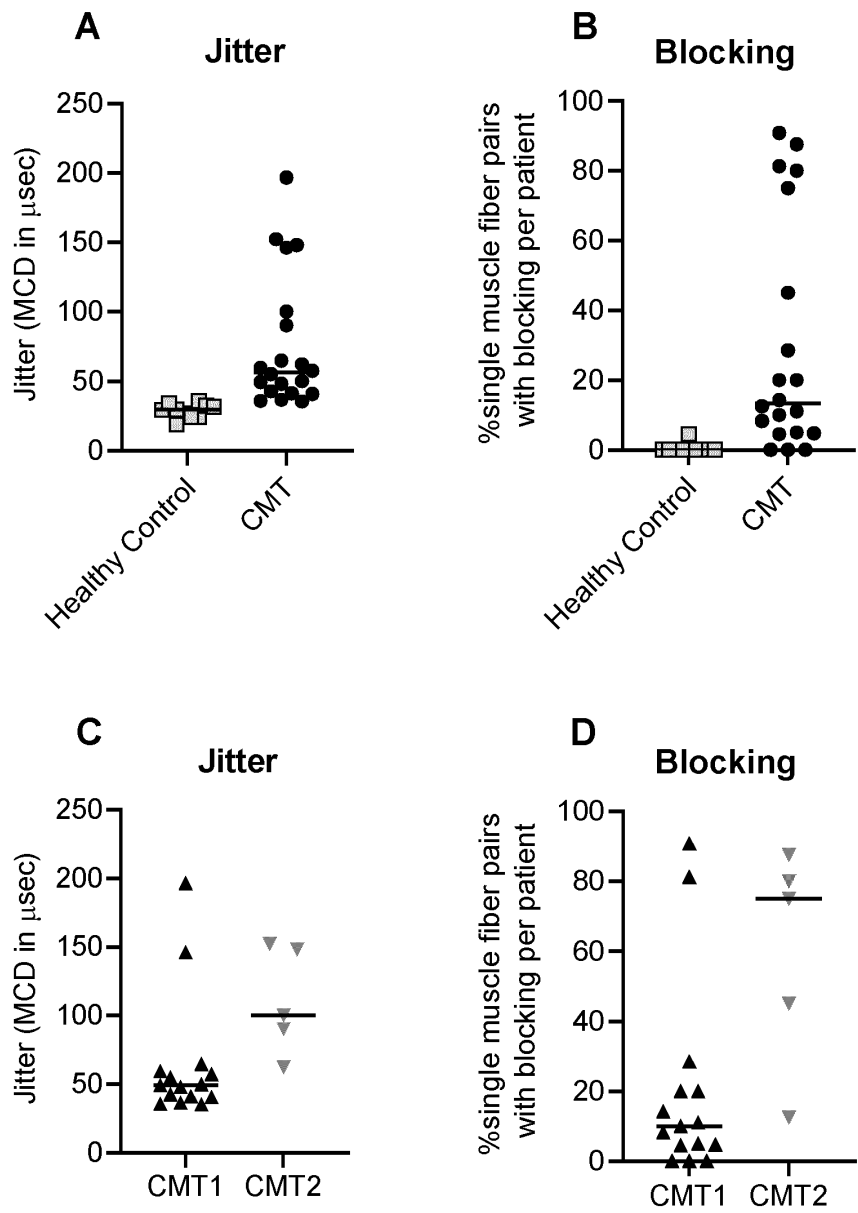


Fig. 5

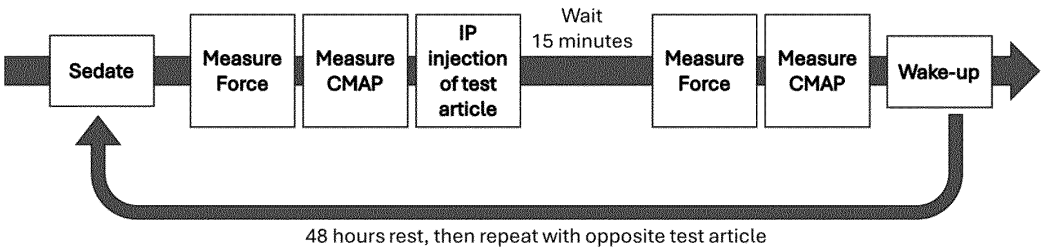


Fig. 6

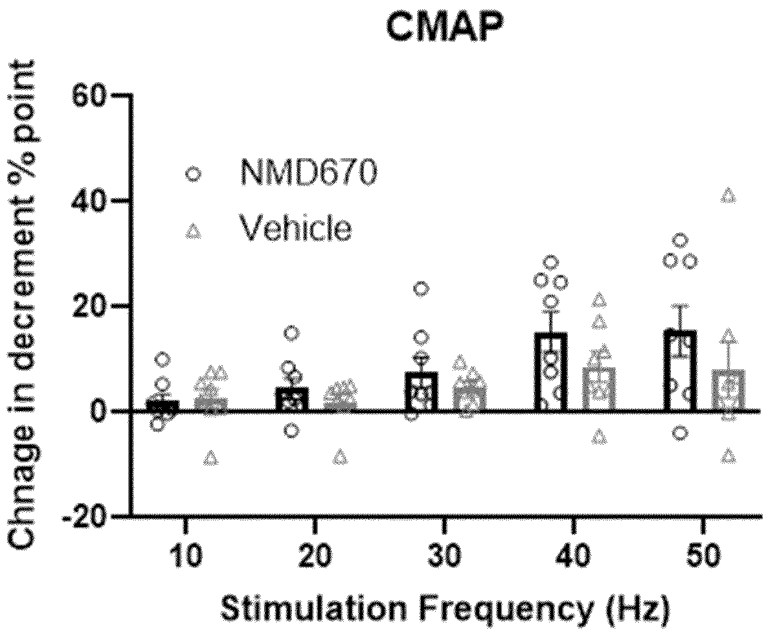


Fig. 7

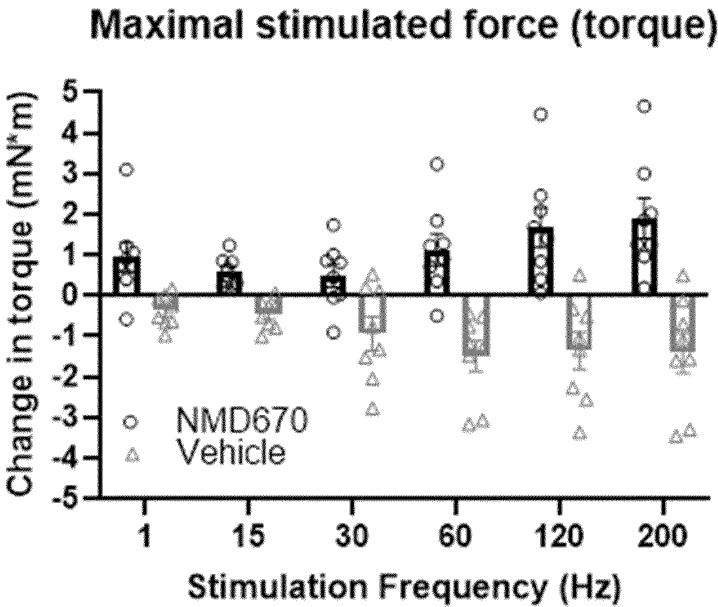


Fig. 8

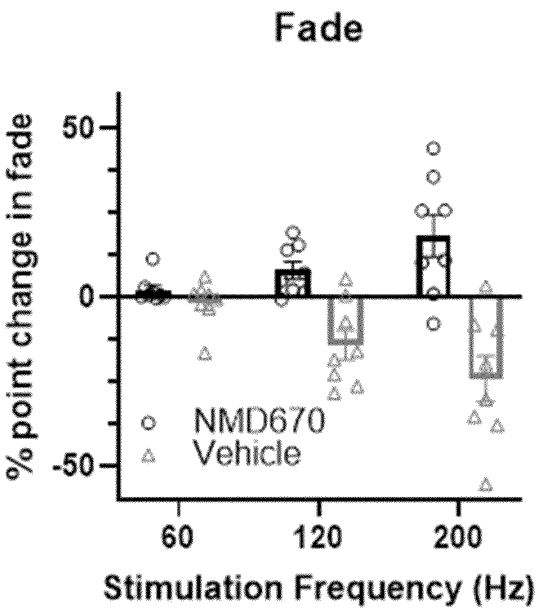


Fig. 9

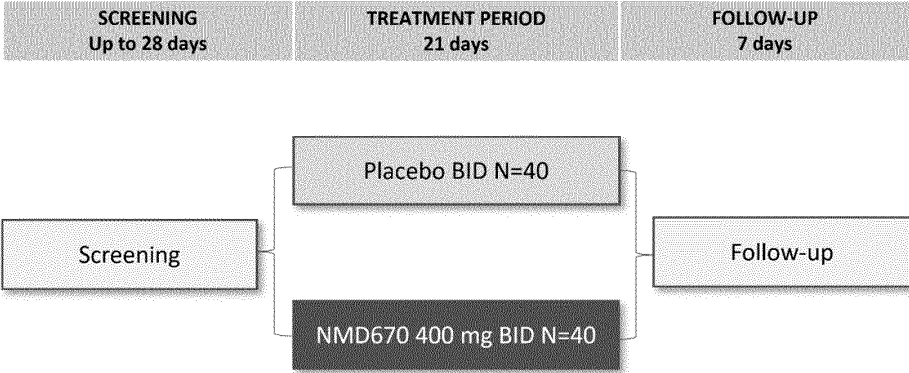


Fig. 1

Procedure	Screening ¹	Baseline	1-Week Treatment	2-Week Treatment	EOT	FU (EOS)		Notes
						V5	V6	
Study Visit	V1	V2	V3	V4	V5	28 (7±2 days After the Last Dose)		
Study Day ± Visit Window	-28 to -1	-1	7±1	14±2	21+3/-1			
Informed consent	X							
Eligibility criteria	X	X ²						
Demography	X							
Height	X							
Weight	X	X			X			
Medical/surgical history	X							
Current medical condition	X							
Substance use/drug screen	X							
Serum pregnancy test	X							WOCBP only
Urine pregnancy test		X			X		X	WOCBP only
Test for follicle- stimulating hormone and oestradiol	X							As needed in women of non-childbearing potential only
HIV and hepatitis B and C tests	X							
Physical examination including neurological, cardiovascular, respiratory, gastrointestinal, musculoskeletal, and skin systems	X						X	
Physical examination including neurological and musculoskeletal systems		X	X	X	X			

Fig. 11A

Procedure	Screening ¹ V1	Baseline V2	1-Week Treatment V3	2-Week Treatment V4	EOT V5	FU (EOS) V6 28 (7±2 days After the Last Dose)	Notes
Study Visit	-28 to -1	-1	7±1	14±2	21+3/-1		
12-lead ECG	X	X	X	X	X	X	
Ophthalmological examinations	X					X	
Clinical chemistry ³ , haematology, and haemostasis	X	X	X	X	X	X	See Appendix 2 for an overview of the protocol-required laboratory tests.
Routine urinalysis	X	X	X	X	X	X	See Appendix 2 for an overview of the protocol-required urine laboratory tests.
Skeletal muscle troponin I, type II exploratory biomarker	X	X	X	X	X	X	
AE/SAE review	X	X	X	X	X	X	
C-SSRS baseline/screening	X	X	X	X	X	X	
C-SSRS since last visit/call		X	X	X	X	X	Will be assessed at all clinic visits and at home every day during the treatment period
Myotonia Behaviour Scale	X	X	X	X	X	X	
Concomitant medication review	X	X	X	X	X	X	
Vital signs	X	X	X	X	X	X	
PK blood sample			X		X		On day 7 (V3): 1 PK sample any time during the visit. On day 21 (V5): 2 PK samples; 1 sample pre-second dose (trough) and 1 sample 2 to 4 hours post-second dose (C _{max})

Fig. 11B

Procedure	Screening ¹ V1	Baseline V2	1-Week Treatment V3	2-Week Treatment V4	EOT V5	FU (EOS)		Notes
						V6	28 (7±2 days After the Last Dose)	
Study Visit								
Study Day ± Visit Window	-28 to -1	-1	7±1	14±2	21+3/-1			
In-clinic study IMP (afternoon dose)			X		X			
SF-36	X	X	X	X	X	X		
CMT-HI	X	X	X		X	X		
Ankle dorsiflexor strength (manual muscle testing)	X							
6MWT	X	X	X		X	X		Approximately 2 hours after the afternoon dose at V3 and V5
10-metre walk/run test	X	X	X		X	X		
TUG	X	X	X		X	X		
CMT-FOM	X	X	X		X	X		Do not repeat 6MWT or TUG items.
ONLS		X	X		X	X		
sfEMG		X			X			Optional and at selected sites only
Randomisation		X						
Dispensing in IRT		X	X		X			With dosing diary
Study IMP			NMD670 400 mg OR placebo twice a day for 21 days					Dosing at home except for afternoon doses on day 7 and day 21
Compliance check			X		X			Diary and per returned dose

Fig. 11C