Liposomal GM1 Formulation Talineuren in Parkinson's Disease: The Open-label Single-arm Interventional Phase I Trial NEON

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Background

- Monosialotetrahexosylganglioside (GM1) is a promising molecule with neuroprotective and immunomodulating properties.
- In Parkinson's disease (PD), Treatment with GM1 has yielded encouraging results both in preclinical models and a randomized placebo-controlled trial.

Talineuren (TLN): Liposomal formulation of GM1, has been designed to improve GM1 biodistribution and reduce administration frequency.

NEON Study Objectives

ClinicalTrials.gov: NCT04976127

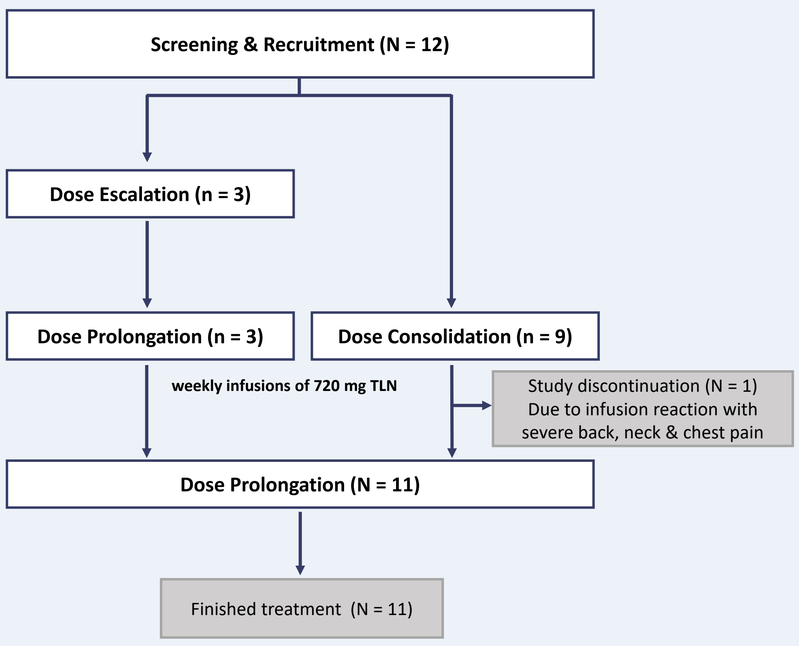
Primary Objectives:

- **Safety**: Occurrence of adverse events (AEs) and serious adverse events (SAEs)

Secondary Objectives:

- **Pharmacokinetic** data of total plasma GM1 after first infusion
- **Preliminary Efficacy** (MDS-UPDRS "on" and "off" scores)

Study Design

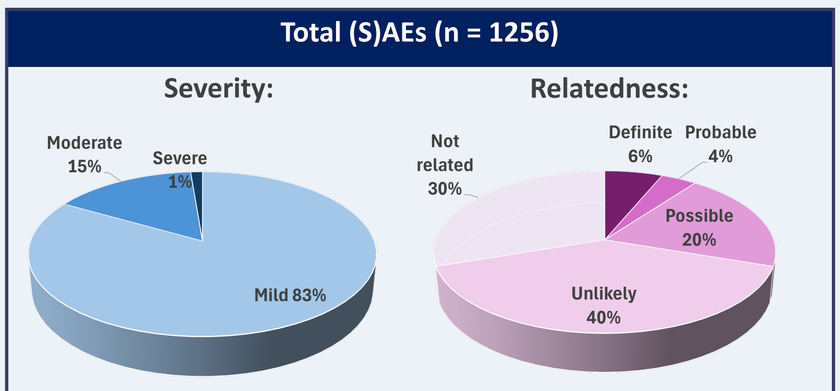


Demographics and Characteristics	Overall N = 12	Dose Escalation n = 3	Dose Consolidation n = 9
Age at registration (years)			
Median (range)	64.5 (46.0–75.0)	63.0 (46.0–68.0)	65.0 (51.0–75.0)
Sex - Female, n (%)	6 (27 %)	1 (33 %)	2 (22 %)
Sex – Male, n (%)	16 (73 %)	2 (67 %)	7 (78 %)
Height (cm)			
Median (range)	172.0 (159.0–188.0)	174.0 (159.0–182.0)	172.0 (160.0–183.0)
Weight (kg)			
Median (range)	77.5 (44.0–108.0)	88.0 (60.0–99.0)	78.0 (64.0–99.0)
BMI (kg/m²)			
Median (range)	25.3 (17.9–33.3)	26.6 (23.7–32.7)	25.8 (21.6–30.8)
Hoehn and Yahr stage at baseline, off medication (Standard of Care), n (%)			
Stage 0	0 (0 %)	0 (0 %)	0 (0 %)
Stage 1	0 (0 %)	0 (0 %)	0 (0 %)
Stage 1.5	5 (23 %)	1 (33 %)	1 (11 %)
Stage 2	16 (72 %)	2 (67 %)	7 (78 %)
Stage 2.5	1 (5 %)	0 (0 %)	1 (11 %)
Hoehn and Yahr stage at baseline, on medication (Standard of Care), n (%)			
Stage 0	1 (5 %)	0 (0 %)	0 (0 %)
Stage 1	7 (32 %)	2 (67 %)	1 (11 %)
Stage 1.5	6 (27 %)	0 (0 %)	4 (44.5 %)
Stage	8 (36 %)	1 (33 %)	4 (44.5 %)
Stage 2.5	0 (0 %)	0 (0 %)	0 (0 %)
Drugs			
L-DOPA, n (%)	19 (86 %)	3 (100 %)	9 (100 %)
Non-ergot-derived dopamine receptor agonist, n (%)	16 (73 %)	2 (67 %)	6 (67 %)
MAO-B Inhibitor, n (%)	7 (32 %)	1 (33 %)	3 (33 %)
COMT inhibitor, n (%)	4 (18 %)	0 (0 %)	3 (33 %)
NMDA agonist, n (%)	2 (9 %)	0 (0 %)	1 (11 %)
LEDD (mg)			
Median (range)	525.0 (160.0–1,275.0)	550.0 (375.0–1,010.0)	750.0 (340.0–1,275.0)

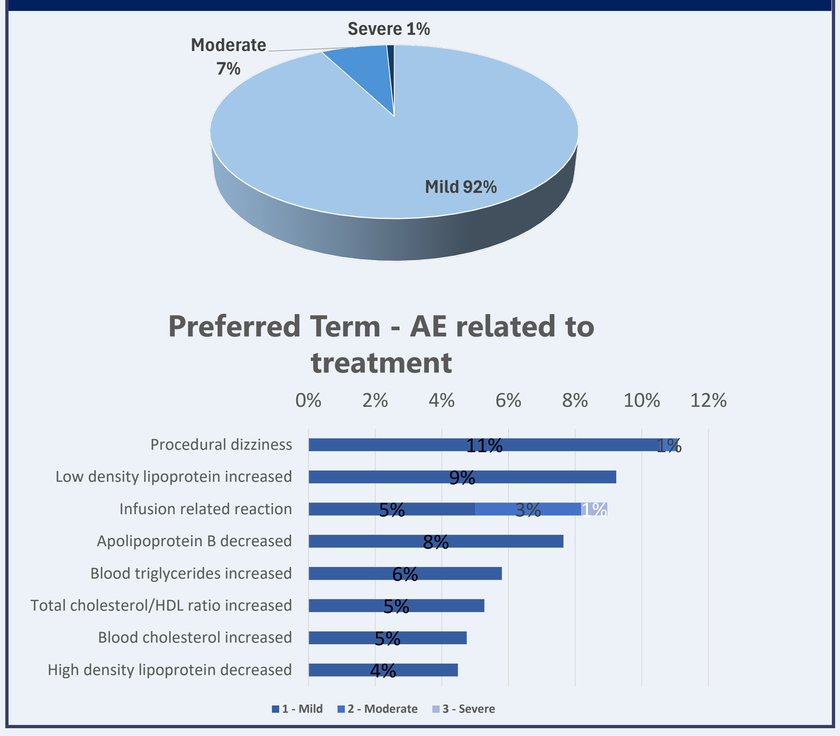
Results

Occurrence of (S)AEs

Total (S)AEs (n = 1256)

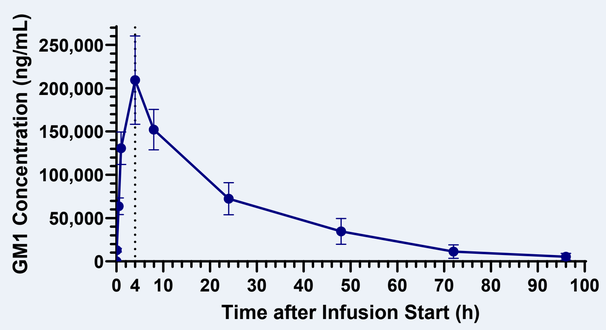


AEs Related to Treatment (n = 379)



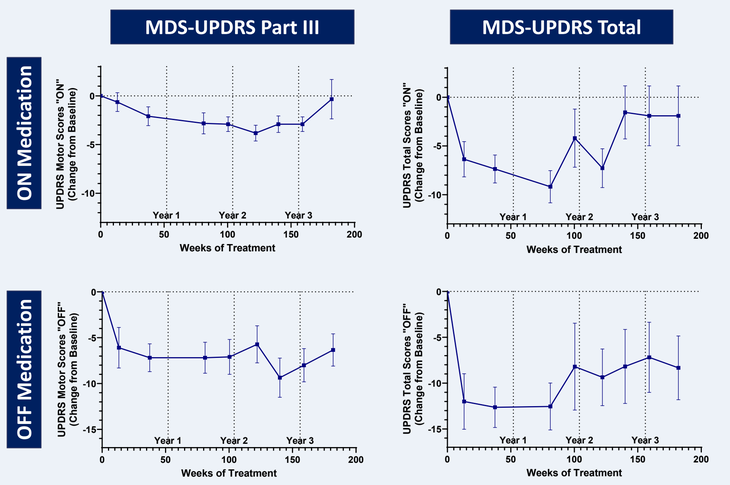
Pharmacokinetics

C_{max} (0.147 mg/mL) reached after 4 h (median)
Median $T_{1/2}$: 12.6 h



Mean (±SEM) GM1 plasma concentration-time profiles after intravenous infusion of 720 mg TLN. N = 9 from the dose consolidation cohort

MDS-UPDRS Scores



N = 11 patients. Mean ± SEM. Mean time in trial since treatment start at up to 138 infusions.

Conclusions

- Weekly intravenous TLN treatment was well tolerated.
- After 8 infusions of the maximal dose of TLN, UPDRS scores ameliorated. This amelioration stabilized and was maintained over 138 infusions/ up 180 weeks.
- Changes in UPDRS scores go along with previous data and highlight the high potential of TLN for PD treatment.

We are looking for scientific and business collaborations!

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References

1. Schneider et al. "A Randomized, Controlled, Delayed Start Trial of GM1 Ganglioside in Treated Parkinson's Disease Patients". *J Neurol Sci*. 2013; 324(1–2): 140–148.
2. Halbherr et al. "Safety and tolerability of intravenous liposomal GM1 in patients with Parkinson disease: A single-center open-label clinical phase I trial (NEON trial)." *PLoS Medicine* 2025; 22(5): e1004472.