

Preliminary Biomarker Findings from the RESCUE-ALS Trial | A Phase 2 Double-Blind, Placebo-Controlled Study of CNM-Au8 to Slow Disease Progression in ALS



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CONCLUSION: CNM-Au8 treatment decreased urinary p75 and plasma UCHL1 levels during the double-blind period; plasma NfL levels were predominantly stable in this early ALS population

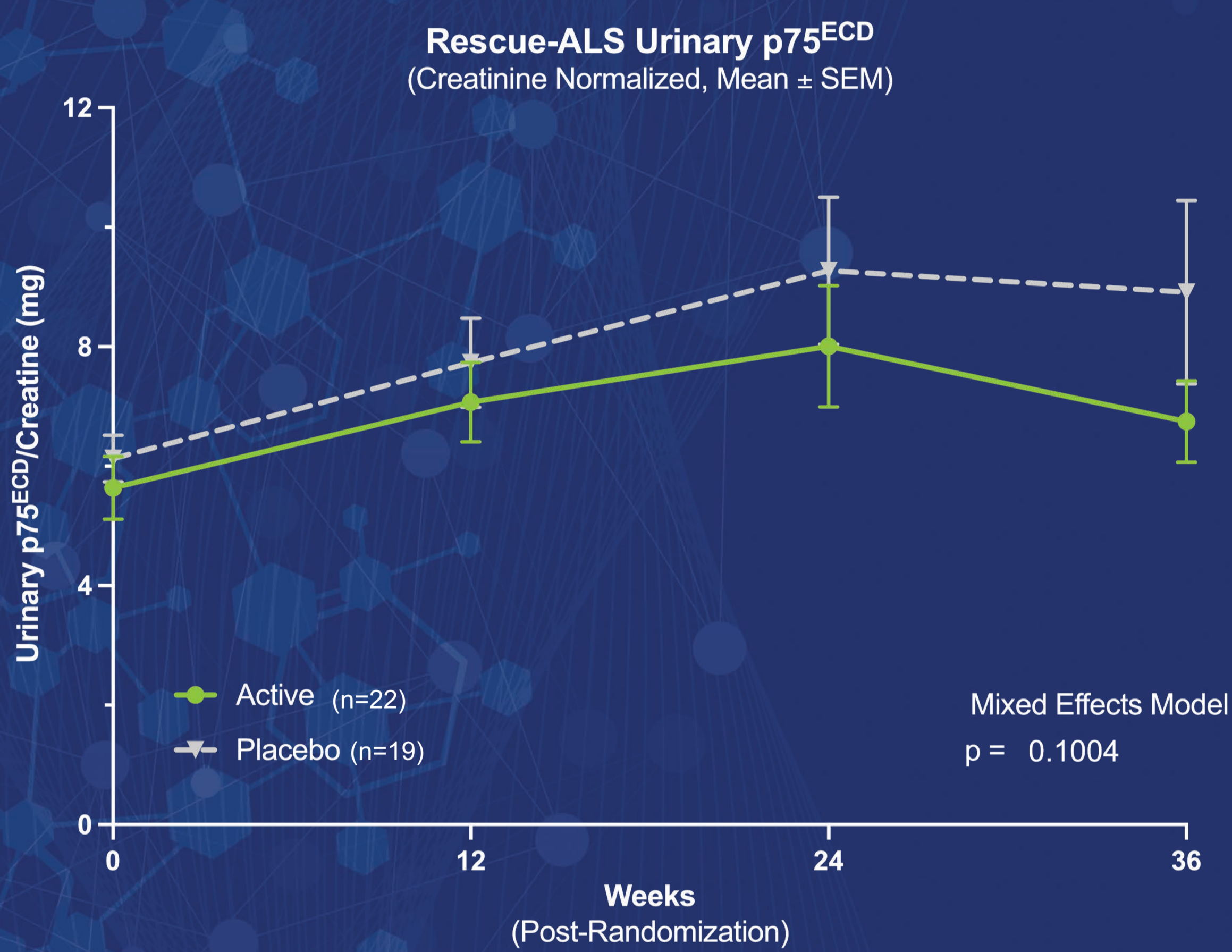
Baseline Demographics

Baseline Value mean (sd)	Age (yrs)	Sex n, (%) Male Female	Onset Site n, (%) Limb Bulbar	Months from Onset	FVC (% pred.)	ALSFRS-R Score	ENCALS Risk Profile ¹	MUNIX Sum
All (n=45)	59.1 (12.3)	M: 26 (58%) F: 19 (42%)	L: 33 (73%) B: 12 (27%)	15.8 (9.3)	81.5 (16.7)	38.7 (6.0)	-4.4 (1.8)	378.2 (175.3)
CNM-Au8 30mg (n=23)	57.0 (13.3)	M: 13 (57%) F: 10 (43%)	L: 16 (70%) B: 7 (30%)	15.5 (7.6)	84.5 (18.3)	38.6 (6.6)	-4.6 (1.7)	380.2 (198.0)
Placebo (n=22)	61.3 (10.9)	M: 13 (59%) F: 9 (41%)	L: 17 (77%) B: 5 (23%)	16.1 (10.9)	78.2 (14.5)	38.8 (5.4)	-4.2 (1.8)	376.2 (152.7)

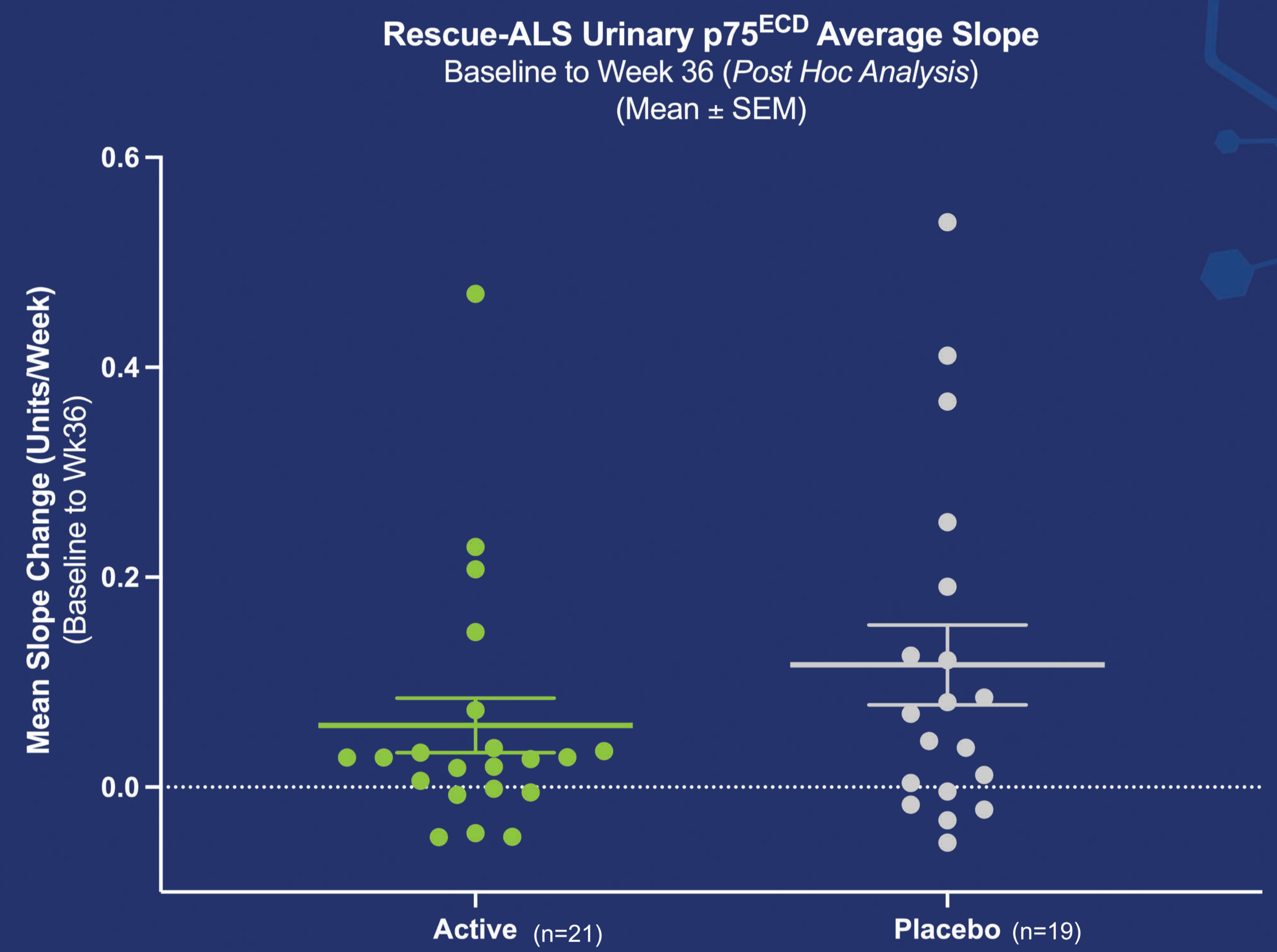
Design Summary

- Early symptomatic ALS
- Randomized (1:1, CNM-Au8 30 mg or placebo)
- 36-week treatment period with open label extension
- 1st EP: MUNIX(4) summed %change of ADM, APB, BB, & TA
- Exploratory pharmacodynamic markers: p75^{ECD}, NfL, UCHL1, GFAP, untargeted proteomics, untargeted metabolomics

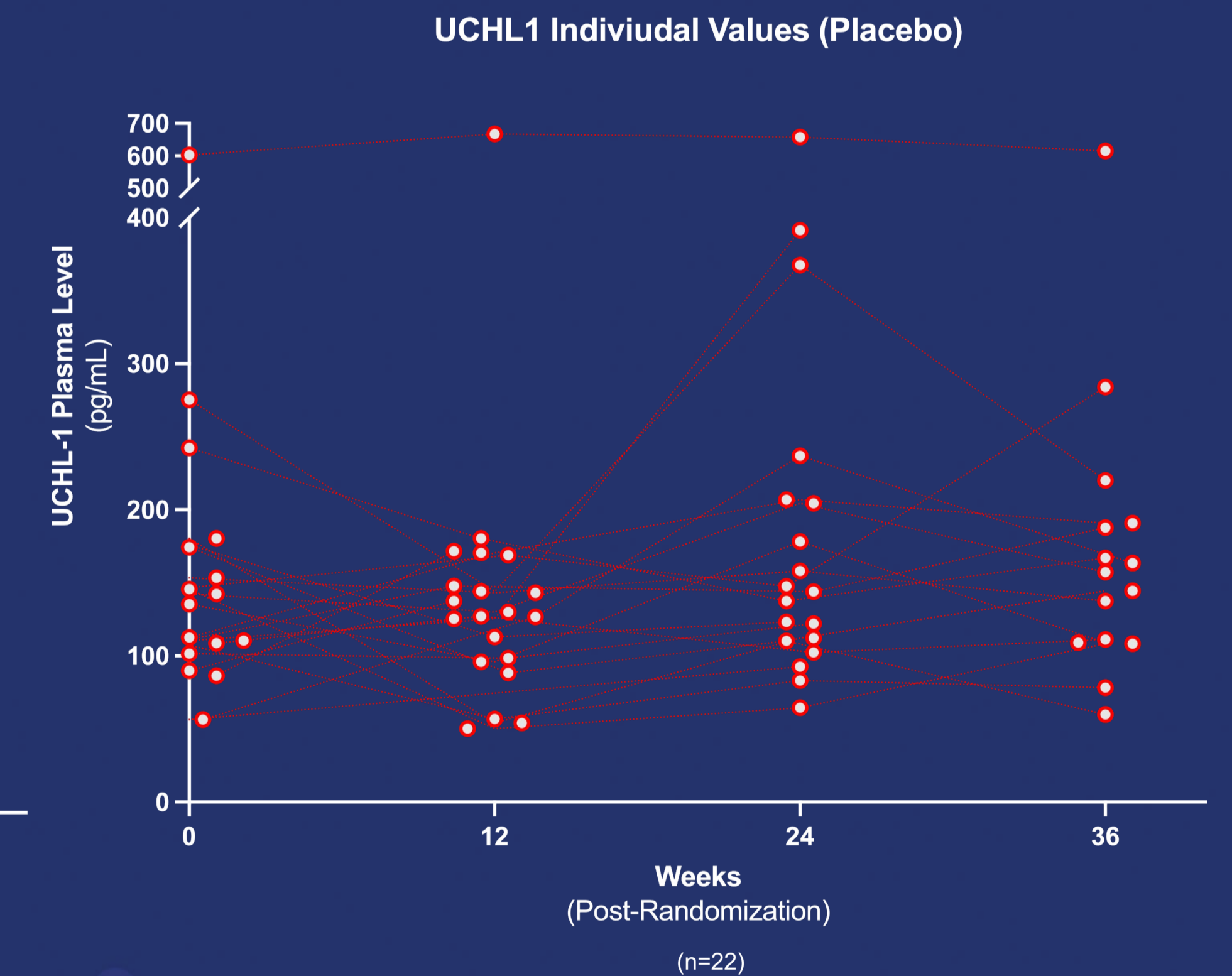
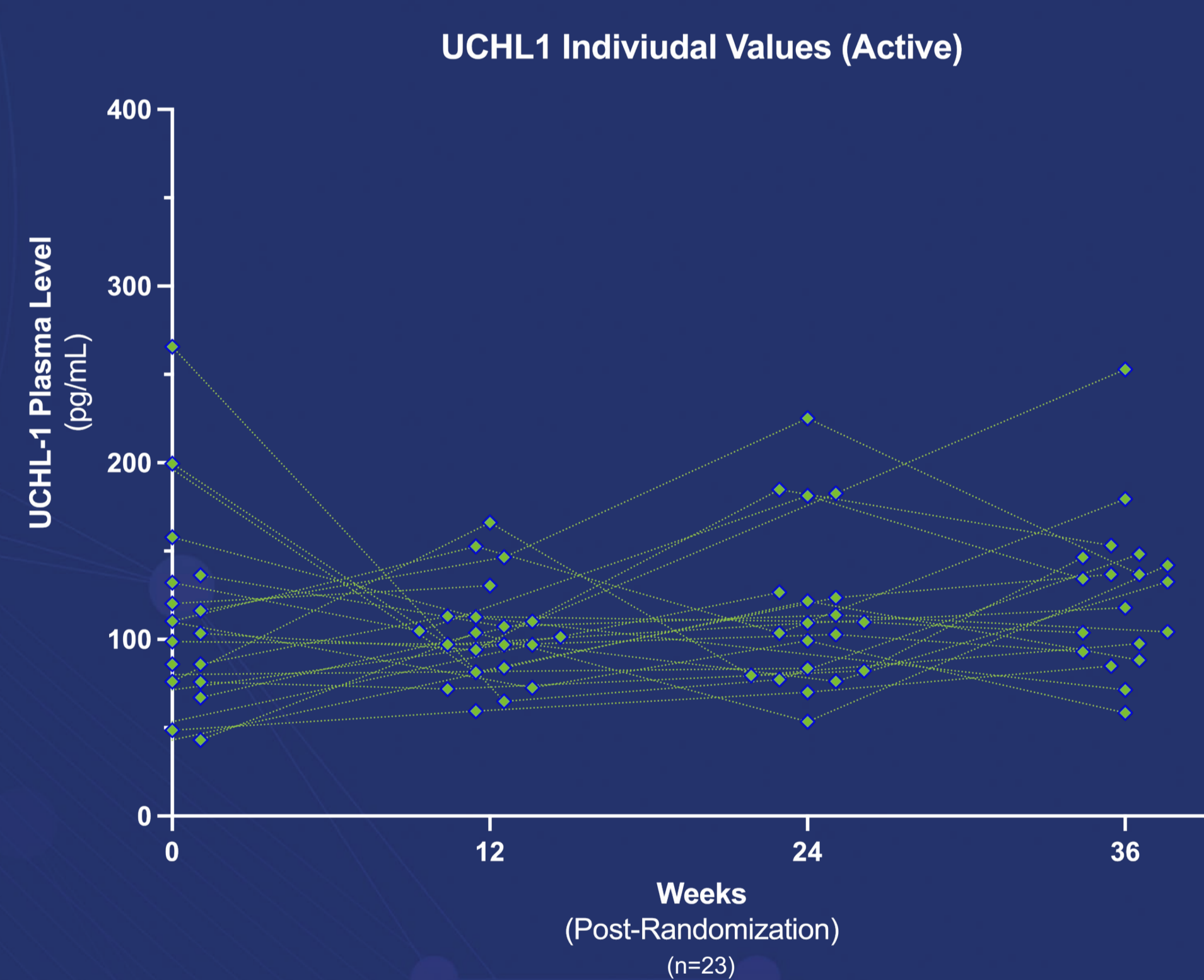
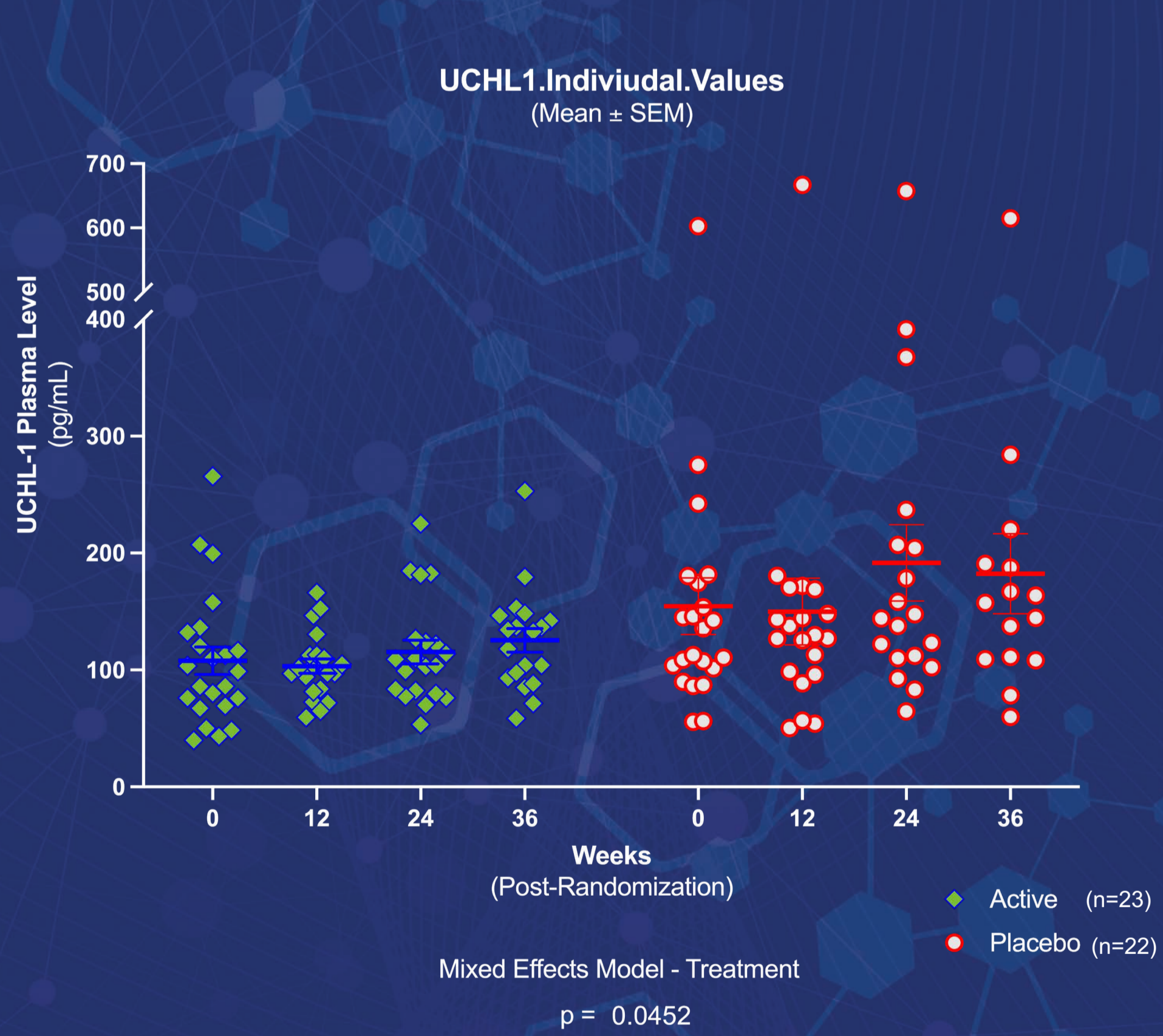
¹Urinary p75^{ECD} Results: CNM-Au8 treatment demonstrated trend for decreased p75^{ECD} (ELISA, M. Rodgers, U. Flinders)



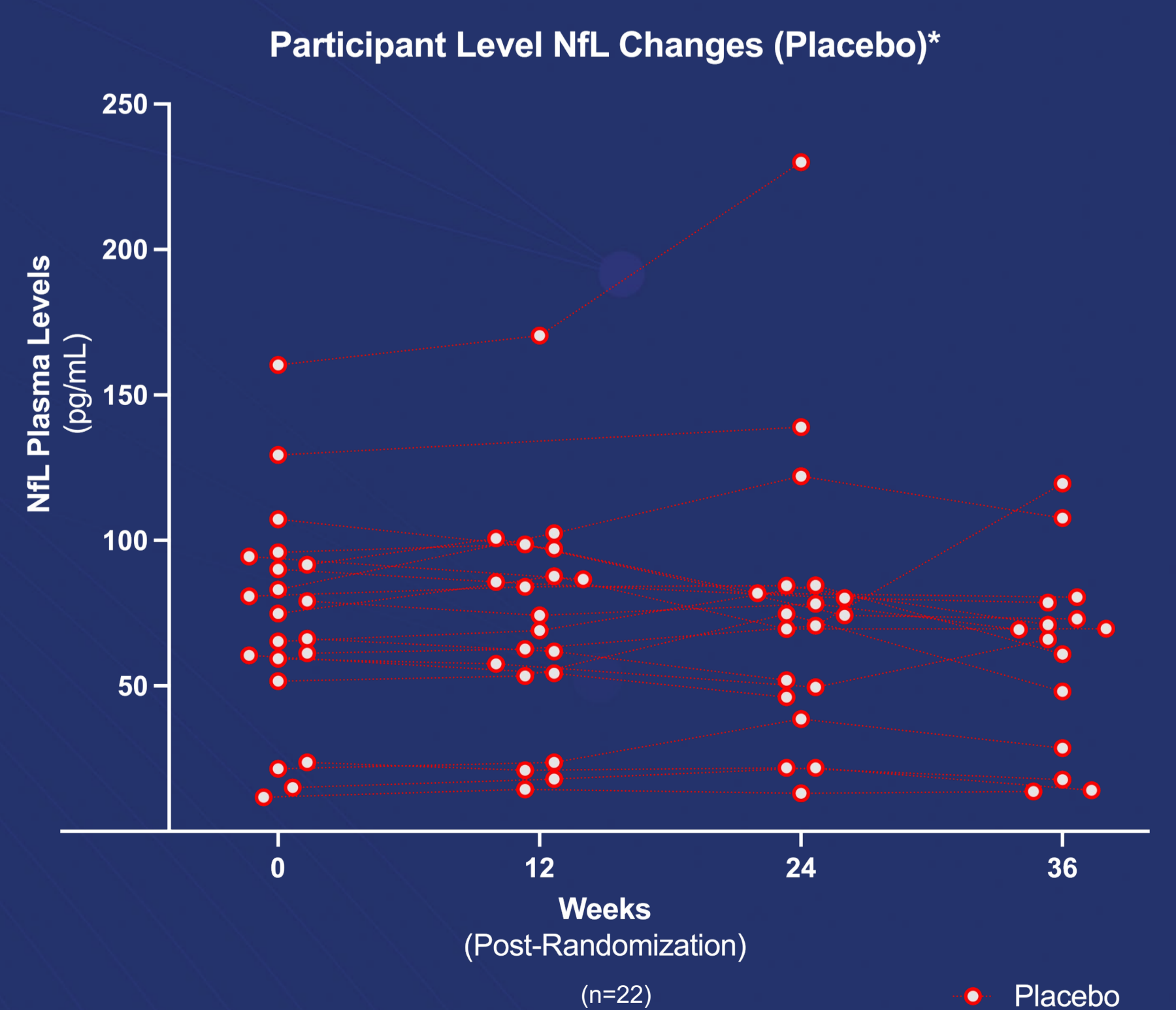
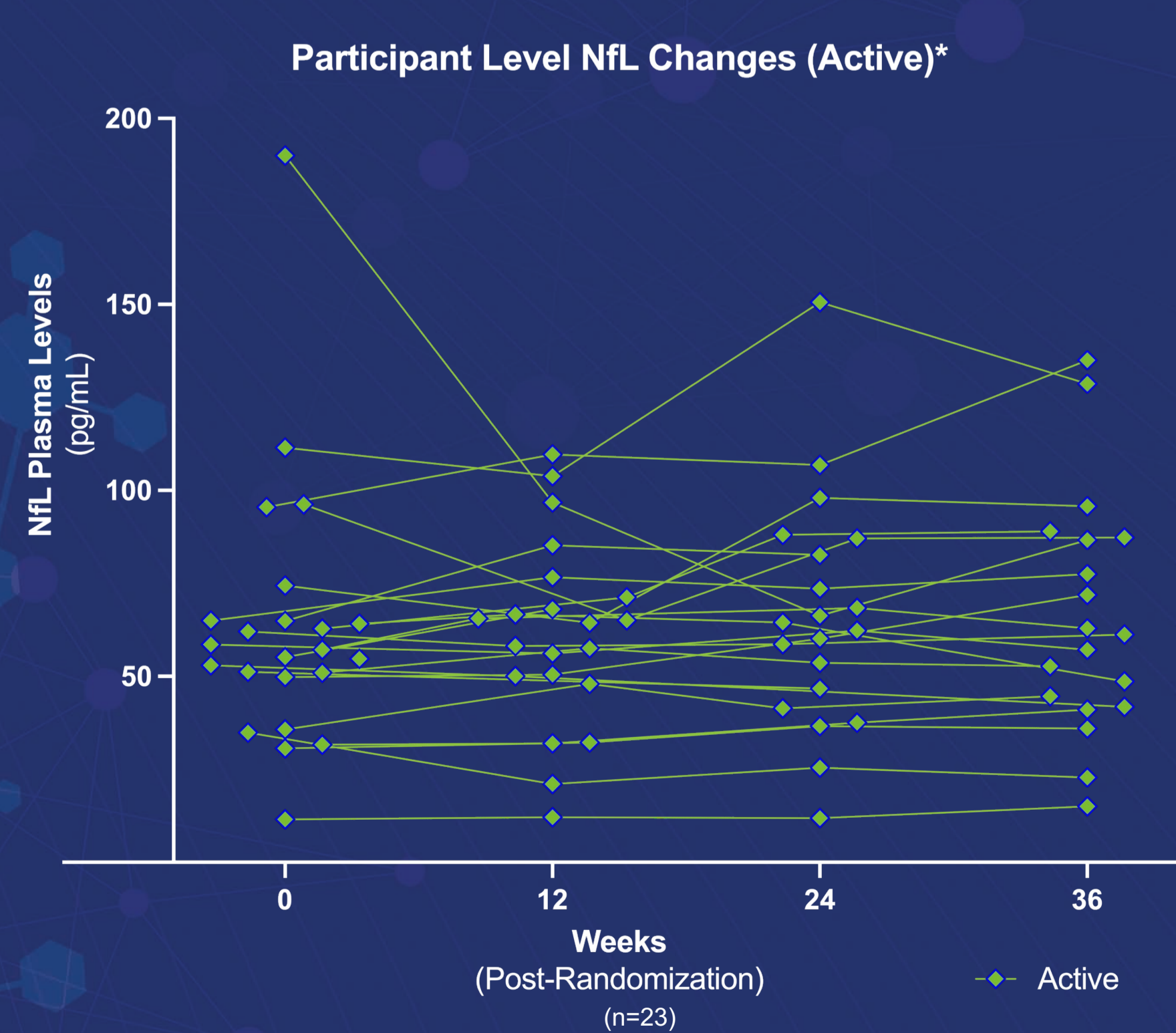
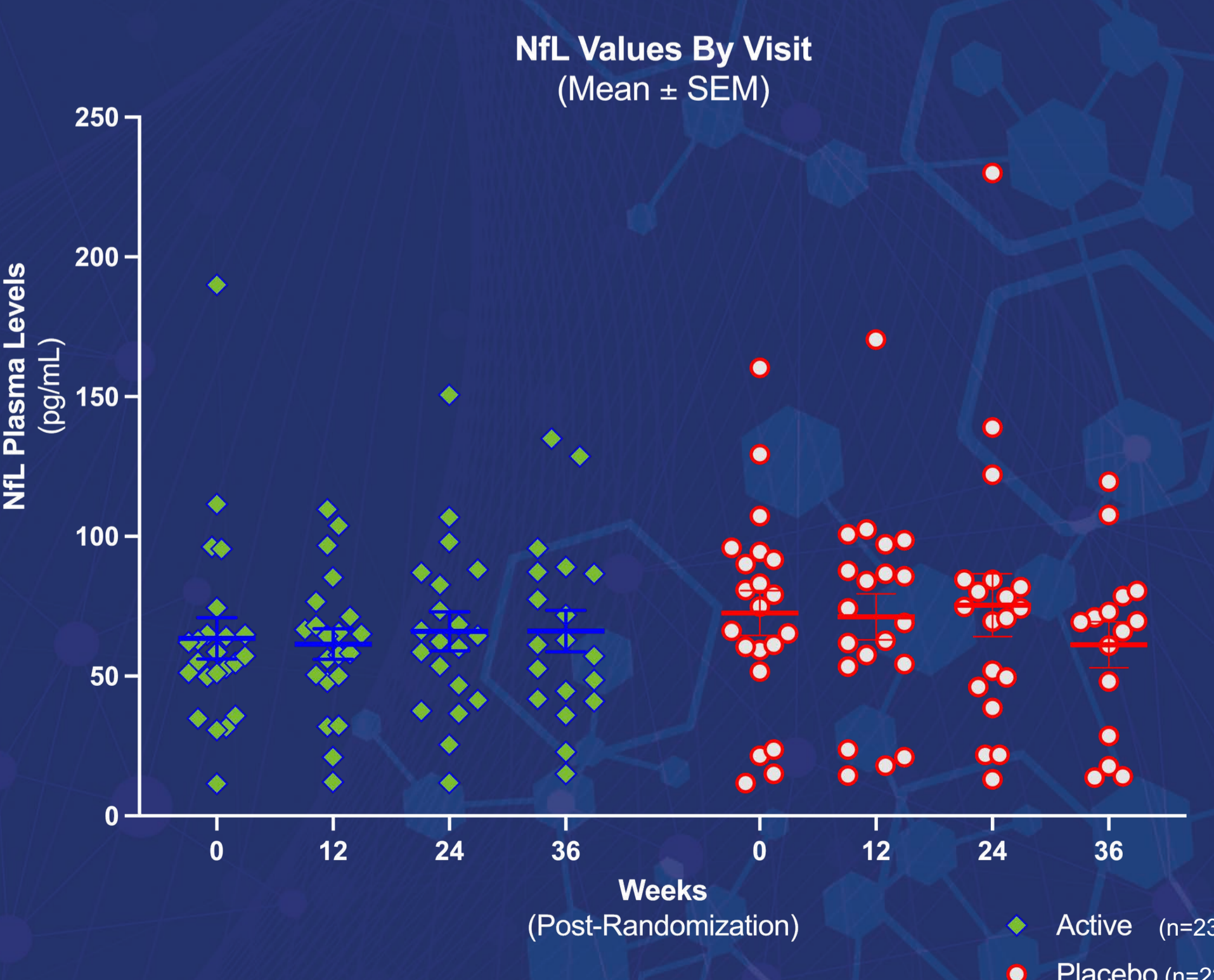
All available data; samples with low creatinine <0.3 mg/ml urinary creatinine.



²Plasma UCHL1 Results: CNM-Au8 treatment blunted increased UCHL1 vs. placebo (Quanterix, Simoa Assay)



Plasma NfL Results: Levels were predominantly stable across both treatment groups (Quanterix, Simoa Assay)



*No adjustments or imputation for missing data

*Missing values are not imputed

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Acknowledgements: We thank the ALS study patients and their families for their support and willingness to engage in clinical research. We thank the site investigators for their research excellence and dedication to patients. We thank FightMND of Australia for substantially funding the RESCUE-ALS trial.

1. Sheapheard et al. 2020 Neurology 2017, 88 (12), 1137-1143. 2. Li et al. 2020 Ann. Clin. Transl. Neurol. 2020, 7 (8), 1420-1428.

