RESCUE-ALS Trial Results: A Phase 2, Randomized, Double-Blind, Placebo-Controlled Study of CNM-Au8 to Slow Disease Progression in ALS

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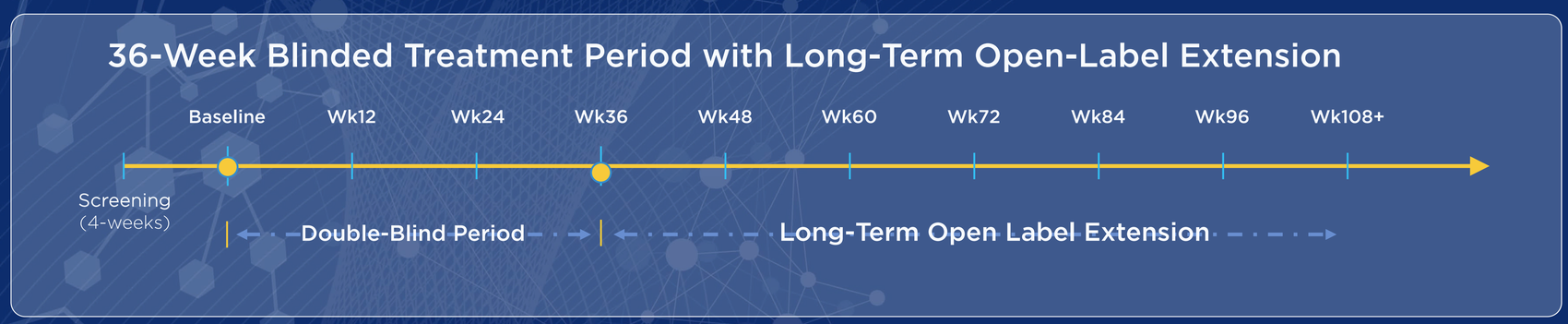
CONCLUSION: RESCUE-ALS has established safety and suggested efficacy of CNM-Au8, a cellular energetic catalyst, for the treatment of ALS

Design Scheme



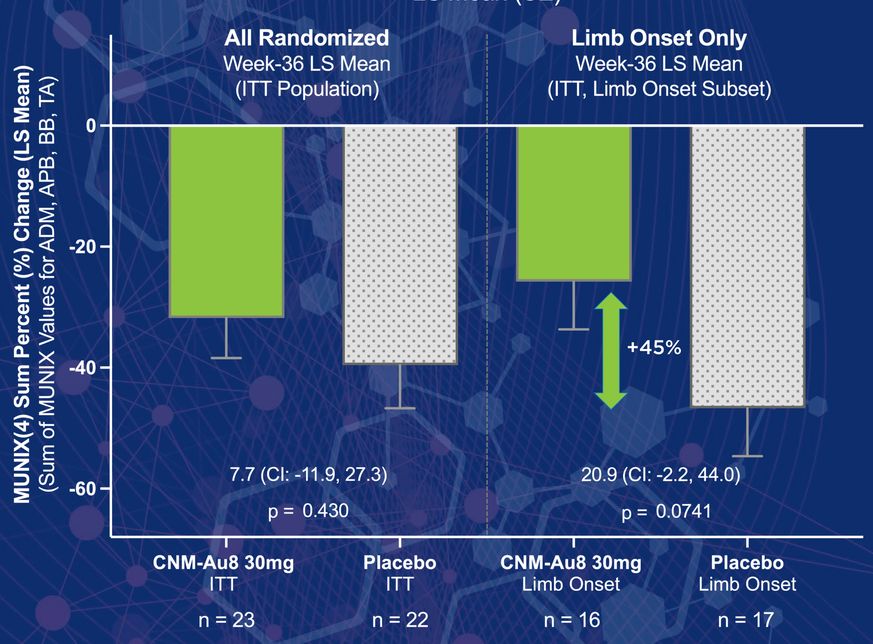
Design Summary

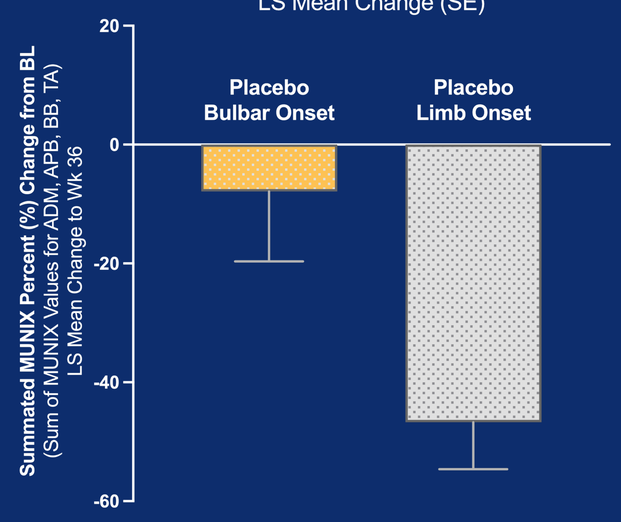
- Early symptomatic ALS
- Randomized (1:1, CNM-Au8 30 mg or placebo)
- 36-week treatment period with open label extension
- 1st EP: MUNIX(4) summed %change of ADM, APB, BB, & TA
- 2nd EPs: absolute MUNIX change, % FVC
- Exploratory EPs: disease progression, 6-pt decline in ALSFRS-R, ALSSQOL-SF, & other neurophysiology endpoints

Baseline Demographics

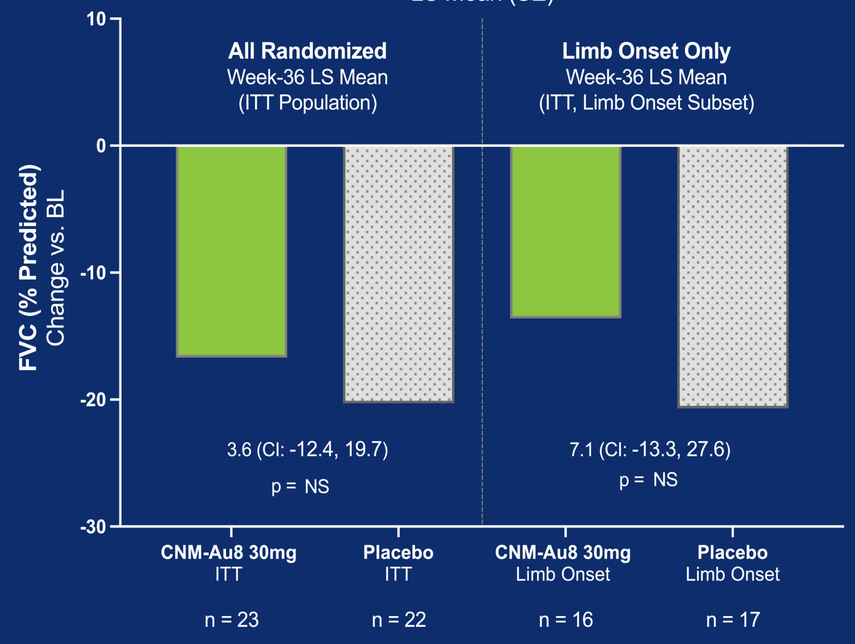
Baseline Value mean (sd)	Age (yrs)	Sex n, (%) Male | Female	Onset Site n, (%) Limb | Bulbar	Months from Onset	FVC (% pred.)	ALSFRS-R Score	ENCALS Risk Profile[1]	MUNIX Sum
All (n=45)	59.1 (12.3)	M: 26 (58%) F: 19 (42%)	L: 33 (73%) B: 12 (27%)	15.8 (9.3)	81.5 (16.7)	38.7 (6.0)	-4.4 (1.8)	378.2 (175.3)
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)

1° Endpoint | Summated MUNIX Change at Week 36





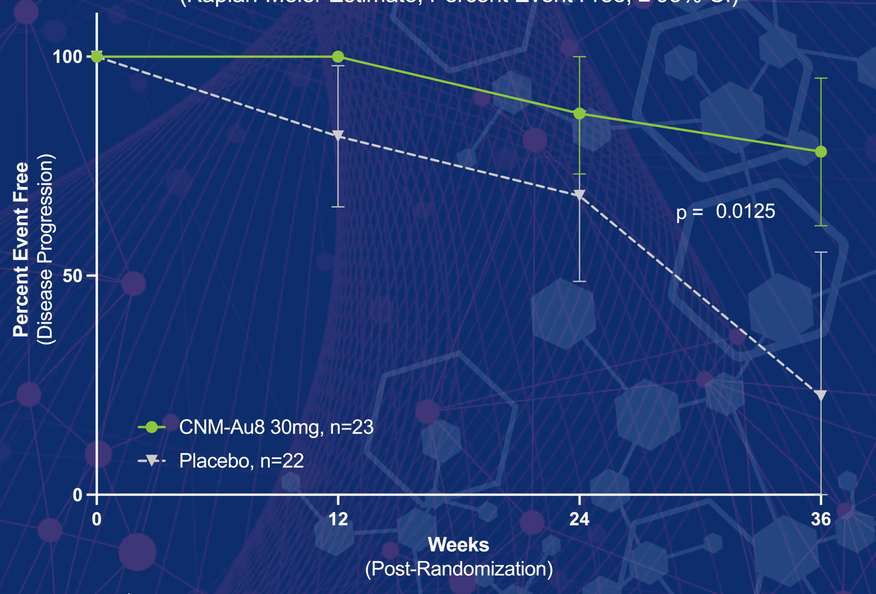
2° EP | FVC Change at Week 36



Primary endpoint p-value is based on mixed model repeat measures with treatment, visit, treatment by visit interaction as fixed effects, and baseline value and ENCALS score as covariates. An unstructured covariance model was used.

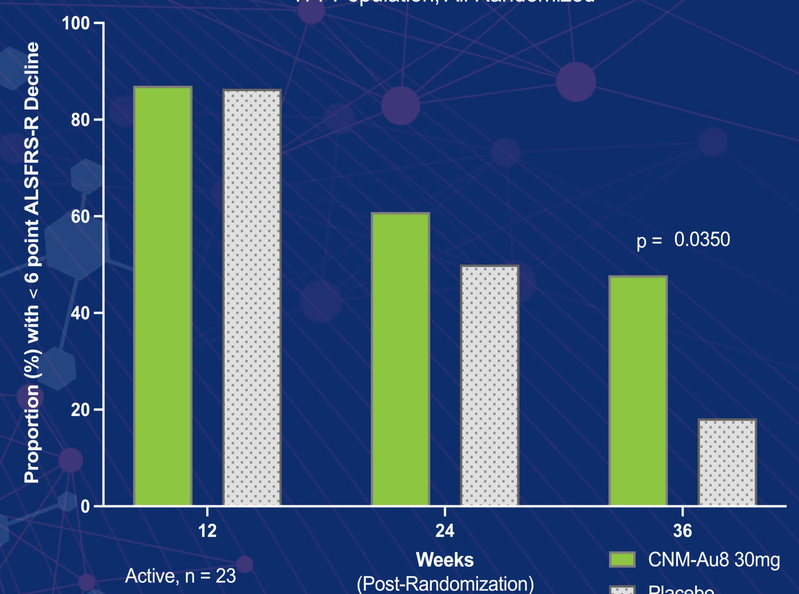
Clinical Endpoints | Exploratory

ALS Disease Progression[1]
RESCUE-ALS Exploratory Endpoint
ITT Population, All Randomized
(Kaplan-Meier Estimate, Percent Event Free, ± 95% CI)



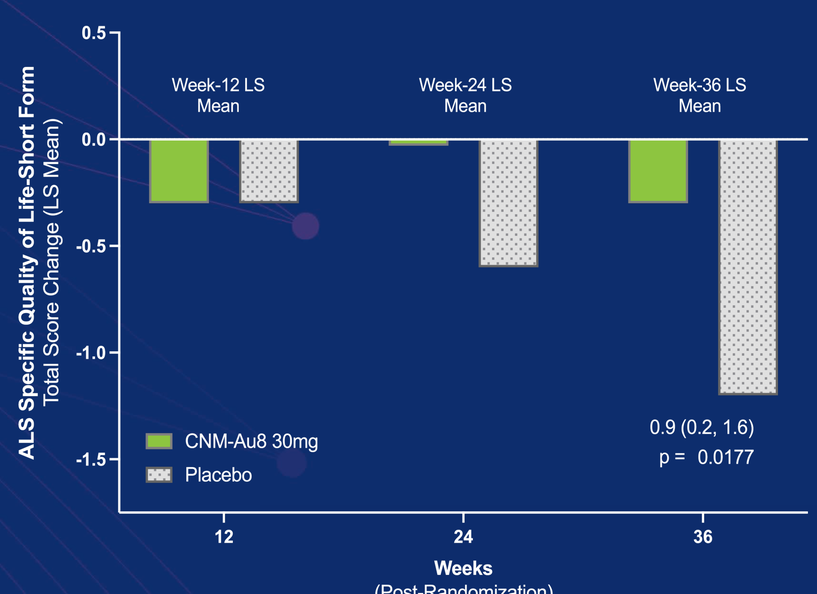
[1] Disease progression defined as death, tracheostomy, or need for non-invasive ventilatory support or gastrostomy tube placement.

ALSFRS-R 6-point Decline Responder
(Proportion free from ≥ 6 point decline)
RESCUE-ALS Exploratory Endpoint
ITT Population, All Randomized



P-value is based on a Chi-Square test

ALS Specific Quality of Life-Short Form Total Score
RESCUE-ALS Exploratory Endpoint
Mixed Model Repeat Measure (ITT Population, All Randomized)
LS Mean Difference



Active, n =	23	23	22
Placebo, n =	21	20	19

P-value is based on MMRM model with treatment, visit, treatment by visit interaction as fixed effects, and baseline value, and ENCALS score as covariates. An unstructured covariance model was used.

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.