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

NCT04098406

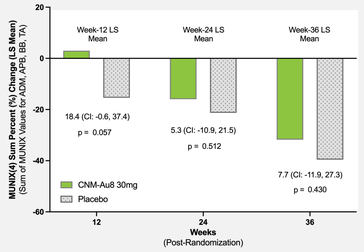
Steve Vucic PhD, DSc, FRACP, FAHMS[1], Parvathi Menon PhD, FRACP[1], William Huynh PhD, FRACP[2], Colin Mahoney, PhD, MB, MRCPI[2], Karen S. Ho, PhD MSc[3], Austin Rynders, RN[3], Jacob Evan[3], Jeremy Evan, PA-C[3], Robert Glanzman, MD FAAN[3], Michael T. Hotchkin[3], Matthew C. Kiernan PhD, DSc, MBBS, FRACP, FAHMS

[1]Concord Repatriation General Hospital, University of Sydney, Australia; [2]Brain and Mind Centre, University of Sydney, Australia; [3]Clene Nanomedicine, Salt Lake City, UT, USA

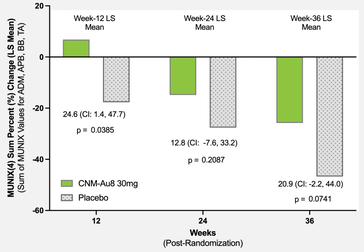
CONCLUSION: RESCUE-ALS has established safety and suggested efficacy of CNM-Au8 for treatment of ALS

Primary EP | Evidence of Lower Motor Neuron Protection

MUNIX

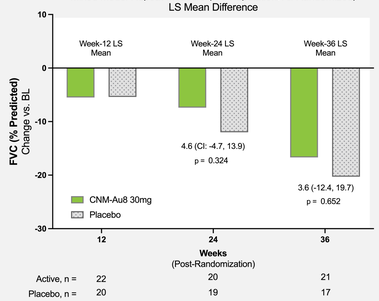


MUNIX – Limb Onset

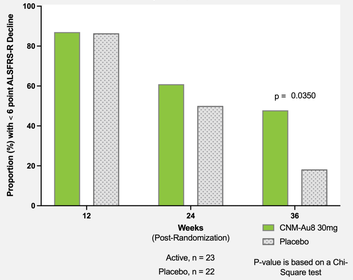


Key Secondary & Exploratory Endpoints | Evidence for Impacting Disease Progression

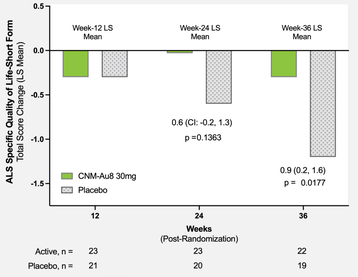
Directional %FVC Benefit



Decreased ALSFRS-R 6pt decline



Improved ALSSSQOL-SF



Design

- 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, & others

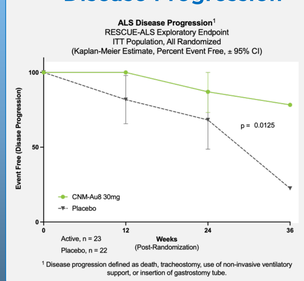
Baseline Demographics & Safety

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 Score
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)

Safety Summary: No CNM-Au8 related SAEs, drug discontinuations, or adverse event (AE) imbalance by system organ class. AEs predominantly mild-to-moderate & transient. The AEs most commonly associated with CNM-Au8 included aspiration pneumonia, n=3; nausea, n=2; abdominal discomfort, n=2.

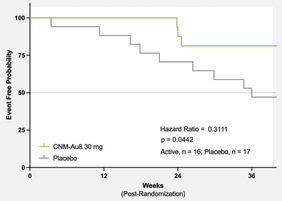
Disease Progression & Observed Survival (vs. ENCALS Predicted)

Disease Progression

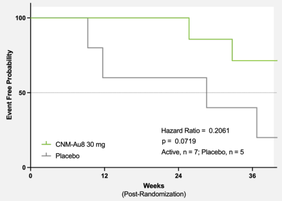


[1] Disease progression defined as death, tracheostomy, use of non-invasive ventilatory support, or insertion of gastrostomy tube.

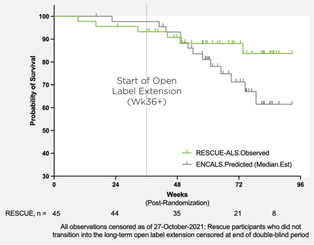
Limb Onset Progression



Bulbar Onset Progression



Observed Survival vs. ENCALS



[1] Disease progression defined as death, tracheostomy, use of non-invasive ventilatory support, or insertion of gastrostomy tube.

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.