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


**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**

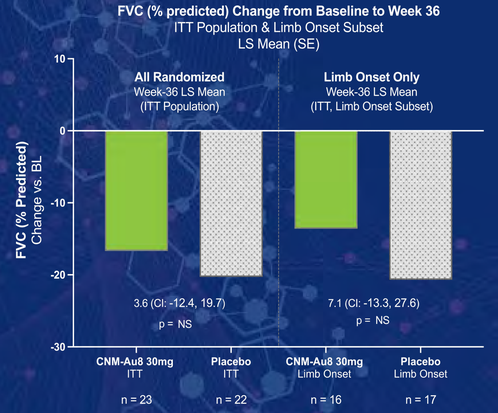
[1]Concord Repatriation General Hospital, University of Sydney, Australia; [2]Brain and Mind Centre, University of Sydney, Australia; [3]Clene Nanomedicine, Salt Lake City, UT, USA


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

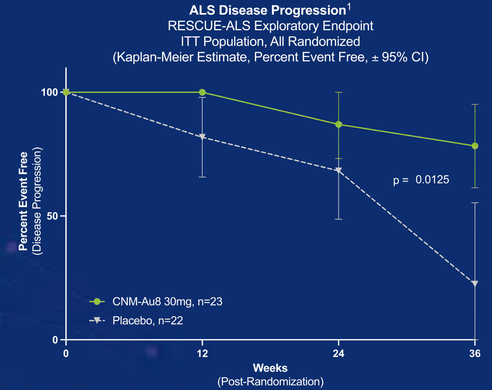
### Baseline Demographics

| 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) |

### 2° EP | FVC Change at Week 36



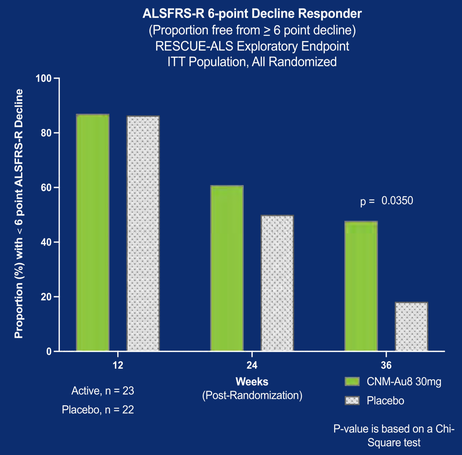

### Clinical Endpoints | Exploratory



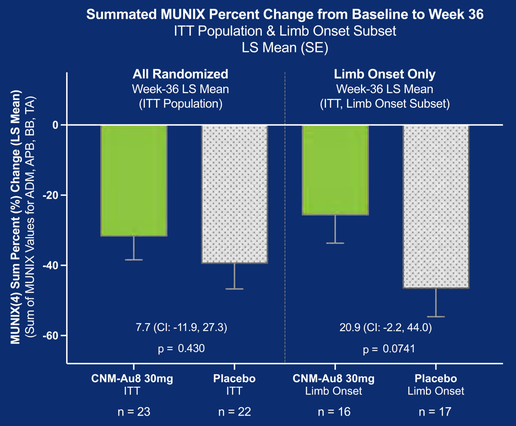

[1] Disease progression defined as death, tracheostomy, or need for non-invasive ventilatory support or gastrostomy tube placement.

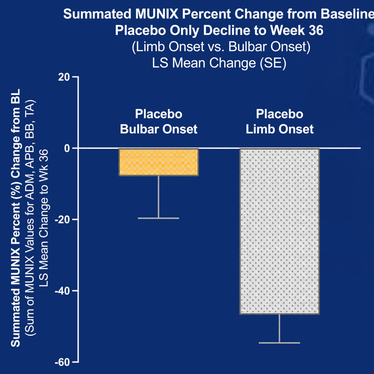
### Design Summary

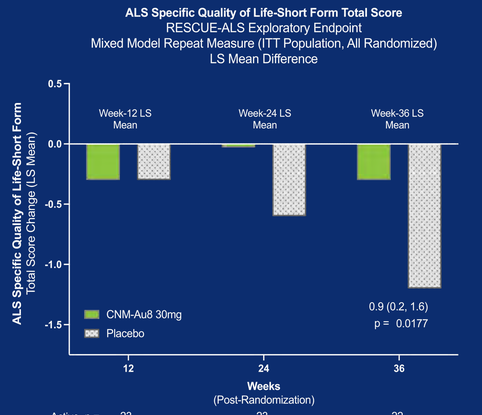
- Early symptomatic ALS
- Randomized (1:1, CNM-Au8 30 mg or placebo)
- 36-week treatment period with open label extension
- 1st EP: MUNIX(4) summed %change of ADM, APB, BB, & TA
- 2nd EPs: absolute MUNIX change, % FVC
- Exploratory EPs: disease progression, 6-pt decline in ALSFRS-R, ALSSQOL-SF, & other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




### 1° Endpoint | Summated MUNIX Change at Week 36




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.

### 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.


### 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.