Design, Objectives, and Preliminary Blinded Data from the Ongoing RESCUE-ALS Trial of CNM-Au8 to Slow Disease Progression in Amyotrophic Lateral Sclerosis Patients

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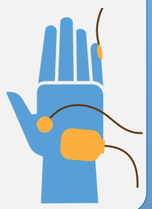
Preliminary blinded efficacy data support a disease modifying potential for CNM-Au8 in the treatment of ALS

Design & Objective

RESCUE-ALS (NCT04098406)

Determine effect of CNM-Au8 on slowing ALS disease progression assessed by electromyography changes

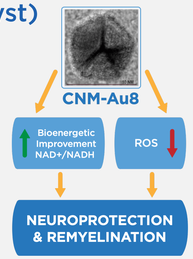
- Early symptomatic ALS patients on stable background therapy (riluzole)
- Randomized (1:1, 30 mg/day active: placebo)
- 36-week treatment period with open label extension
- Electromyography primary endpoint
  - MUNIX(4) change:
    Mean change in average MUNIX values for ADM, APB, BB, & TA



Investigational Product

CNM-Au8 (Nanocatalyst)

- Aqueous suspension of clean surfaced, faceted gold nanocrystals
- Orally administered
- Blood-brain barrier penetrant
- NOAEL toxicity findings
- Well-tolerated Phase 1 Trial
- ***MOA: bioenergetic catalysis for neurorepair and remyelination***



Baseline Demographics [a]

Baseline Values	Subjects n (%)	Age [yrs.] mean (SD)	Time from Diagnosis [Months] mean (SD)	Riluzole Therapy (%)	ALSFRS-R mean (SD)	FVC % mean (SD)
All	44 (100%)	58.7 (12.1)	4.7 (4.6)	89%	38.6 (6.1)	80.8 (16.3)
Female	18 (41%)	59.0	4.8	83%	38.5	80.2
Male	26 (59%)	58.5	4.7	92%	38.7	81.3

[a] All data captured in the EDC as of 27-October-2020.

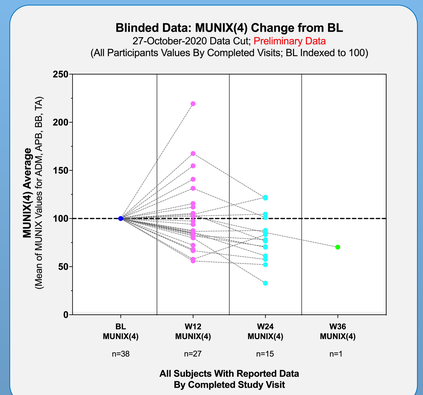
Visit Plan & Endpoints

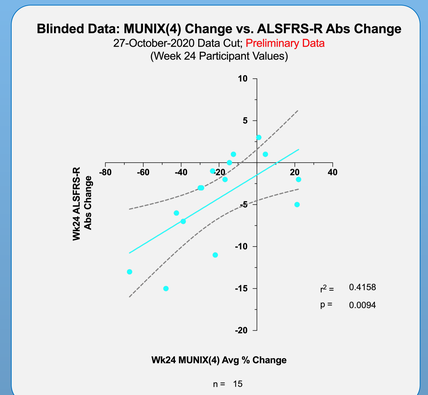
1° **Change in Average Motor Unit Index(4)**
Abductor Digiti Minimi, Abductor Pollicis Brevis, Biceps Brachii, Tibialis Anterior

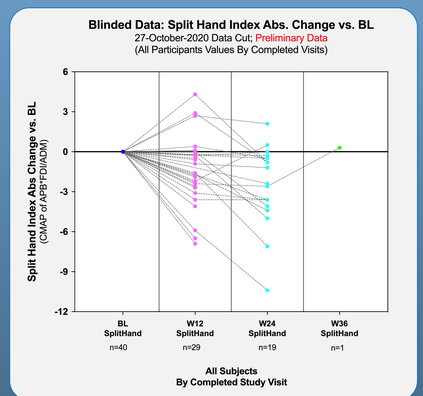
2°
- Split Hand Index
- MScanFit MUNE
- Motor Unit Size Index
- Neurophysiological Index

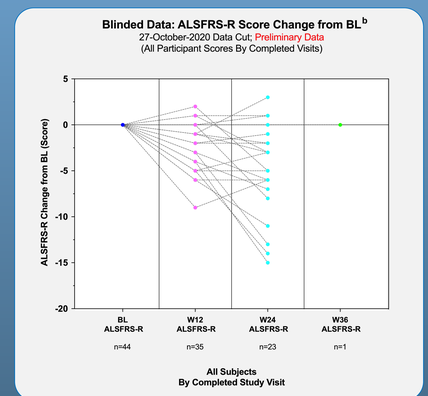
Exploratory Endpoints
- ALSFRS-R
- FVC
- ALSSQOL
- Combined Joint-Rank (Survival + ALSFRS-R)
- Change in Rate of ALSFRS-R progression

Blinded Interim Data









[b] Predominantly "Milder" ALSFRS-R at BL: mean 38.6; median: 39; range: 20 – 47.

Conclusions

- Preliminary *blinded* data demonstrate mean MUNIX(4) improvements or stability in ~50% of enrolled subjects
- Well tolerated safety profile with no reports of related SAEs to date

Acknowledgements

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