CNM-Au8® 30 mg Demonstrated a Significant Survival Benefit Compared to Regimen A Over Long-Term Follow-Up in the HEALEY ALS Platform Trial

HEALEY ALS Platform Trial

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Objective: to investigate the effects of CNM-Au8 30mg on long-term survival compared to concurrent controls randomized into the HEALEY ALS Platform Trial from Regimen A

Conclusion: CNM-Au8 30 mg treatment was associated with a significant survival benefit; the restricted mean survival time (RMST) difference was 124 days (4.1 months, 95% CI: 3–245 days, p=0.044). In the subset with consistent baseline disease severity (sNfL ≥ 33 pg/mL, TRICALS: -6.5 to -2.5), the covariate adjusted RMST difference was 197 days (95% CI: 65 to 329 days, p=0.004)

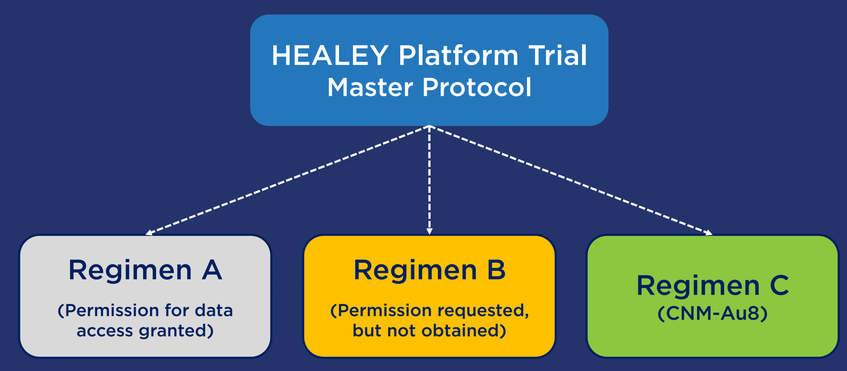
CNM-Au8® Overview | Mechanism of Action

CNM-Au8® is an investigational therapy that improves central nervous system cells' survival and function via a catalytic mechanism of electron exchange that targets mitochondrial function via the nicotinamide adenine dinucleotide (NAD) pathway, while simultaneously reducing oxidative stress

Methods

- All-cause mortality was analyzed *post hoc* by comparing the full analysis set of CNM-Au8 30 mg (Regimen C, n=59) to Regimen A participants (n=162) (i.e., untreated comparator) by restricted mean survival time (RMST) incorporating the HEALEY prespecified covariates for survival analyses
- For Regimen A participants, there was no difference in survival between participants originally randomized to active vs. placebo, supporting use of the full dataset as a comparator
- Survival status up to 48 months post-baseline was captured from public records and site reporting
- Supportive analyses were conducted in the subset of participants with comparable risk (filtering participants based on baseline risk imbalances: serum NfL (sNfL) ≥ 33 pg/mL, TRICALS risk score range of -6.5 to -2.5)

HEALEY ALS Platform Trial
Original Randomization Scheme



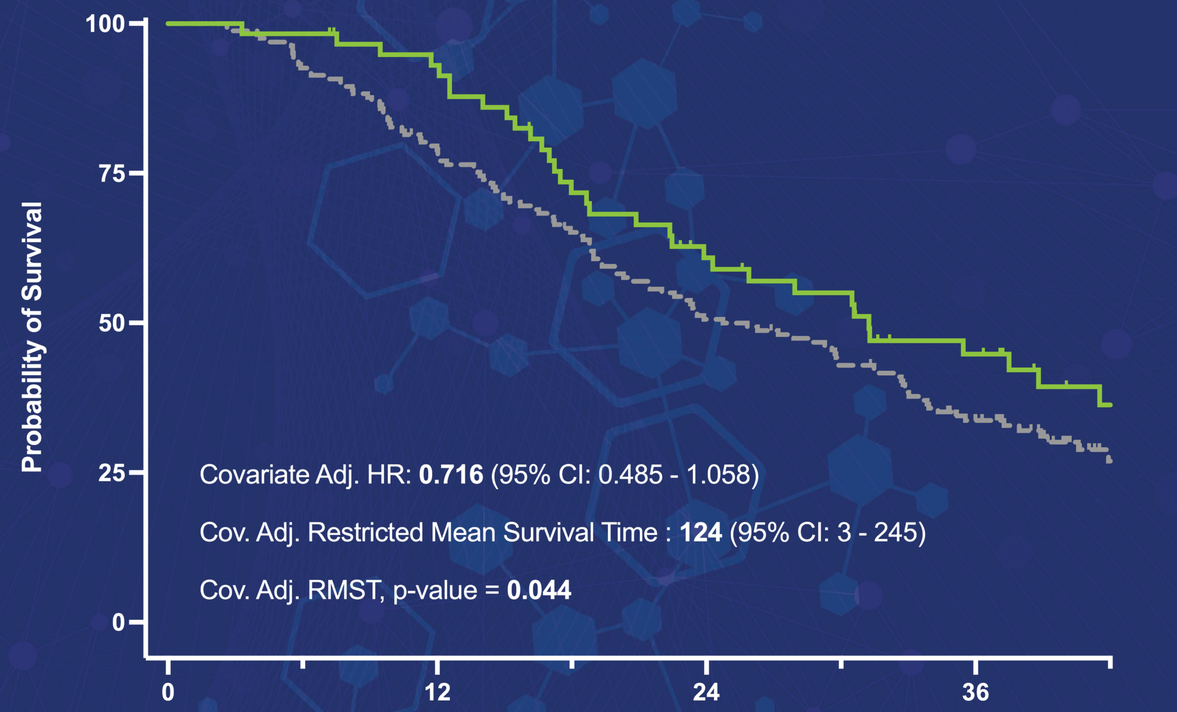
Concurrently Randomized Regimens

- Same Clinical Trial Sites
- Same Master Protocol
- Same Eligibility Criteria
- Same Follow-Up Period

Survival Results

Full Analysis Set (ITT)

HEALEY ALS Platform Trial | Long Term Survival
CNM-Au8 30 mg vs. Regimen A (All)
Kaplan-Meier Estimator | Survival Status (Through Sep-2024)



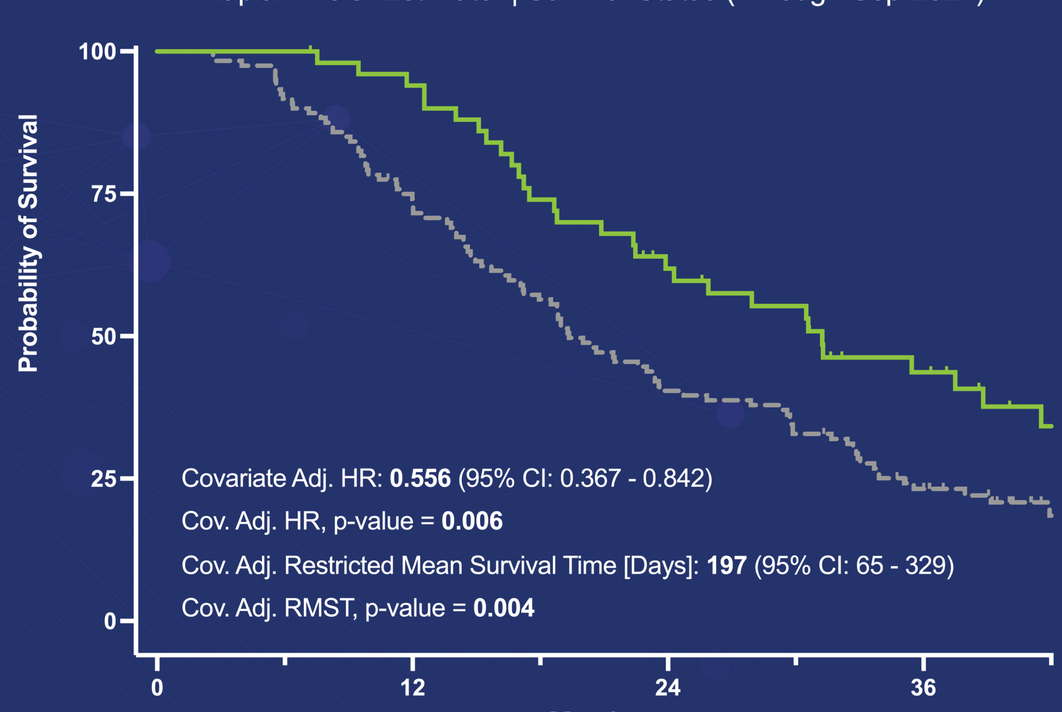
No. At Risk

CNM-Au8 30 mg:	59	58	53	40	32	28	20	12
Regimen A:	162	150	126	103	80	67	45	14

Comparable Risk Population

(sNfL ≥ 33 pg/mL, TRICALS: -6.5 to -2.5)

HEALEY ALS Platform Trial | Long Term Survival
CNM-Au8 30 mg vs. Regimen A (All)
Filtered by (i) BL sNfL ≥ 33 pg/mL, (ii) BL TRICALS Score: -6.5 to -2.5
Kaplan-Meier Estimator | Survival Status (Through Sep-2024)



No. At Risk

CNM-Au8 30 mg:	51	51	47	37	29	25	17	10
Regimen A:	120	110	88	67	48	39	24	8

Acknowledgements: We are indebted to the participants and investigators of the HEALEY ALS Platform Trial whose contributions have led to the conduct of these analyses. Notes: HEALEY survival analyses covariates included: (i) pre-treatment ALSFRS-R slope, (ii) months from symptom onset, (iii) age, (iv) background riluzole treatment, (v) background edaravone treatment.

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