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Neurizon to Present Preclinical and Collaborative Poster Data at 2025 NEALS Annual Meeting

Highlights:

- **Neurizon® Therapeutics to present data at 2025 NEALS Annual Meeting on preclinical characterization of NUZ-001 and its active metabolite, NUZ-001 Sulfone, demonstrating brain penetration and reduction of TDP-43 aggregation — a shared driver of ALS and other neurodegenerative diseases.**
- **A second poster, in collaboration with Berry Consultants, Brigham Young University, and Duke University, explores innovative statistical methodologies using external controls from the PRO-ACT database to enhance early-phase ALS trial design and analysis.**
- **Both presentations reinforce Neurizon's scientific commitment to advancing innovative ALS therapies and strengthening collaboration with global experts to accelerate clinical research.**

10 October 2025 – Melbourne, Australia: Neurizon Therapeutics Limited (ASX: NUZ & NUZOA; OTCQB: NUZTF) ("Neurizon" or "the Company"), a clinical-stage biotech company dedicated to advancing innovative treatments for neurodegenerative diseases, is pleased to announce two scientific presentations at the 2025 NEALS (Northeast Amyotrophic Lateral Sclerosis Consortium) Annual Meeting, held in Clearwater, Florida, October 8-10, 2025.

Poster Presentation 1:

"Preclinical Characterization of NUZ-001: A Brain-Penetrant Small Molecule That Reduces TDP-43 Aggregation in ALS Models"

Authors: Jeffrey M. Brown, Ph.D.; Michael Thurn, Ph.D.

Neurizon will present new preclinical data demonstrating that NUZ-001, its lead investigational therapy for ALS, together with its active metabolite, NUZ-001 Sulfone, achieves therapeutically relevant concentrations in the brain and significantly reduces TDP-43 protein aggregation—a key hallmark of ALS and other neurodegenerative diseases.

Studies in human iPSC-derived motor neurons carrying a TDP-43 mutation (M337V) showed that NUZ-001 and NUZ-001 Sulfone reduced pathological TDP-43 aggregation by up to 75% at brain-relevant concentrations. Pharmacokinetic and selectivity analyses further demonstrated robust brain penetration, sustained plasma levels, and high pharmacological specificity across central nervous system targets.

These findings reinforce the biological rationale for NUZ-001's ongoing clinical development in ALS and support its progression into the upcoming Phase 2/3 HEALEY ALS Platform Trial. They also highlight the potential to expand NUZ-001's therapeutic application beyond ALS to other neurodegenerative diseases driven by TDP-43 and related proteinopathies.

Poster Presentation 2:

"Leveraging External Controls from PRO-ACT for Exploratory Efficacy Assessment in Early-Phase ALS Trials"

Authors: Melanie Quintana, Berry Consultants, Ph.D, Philip White, Department of Statistics, Brigham Young University, Ph.D, Peijin Wang, Department of Biostatistics and Bioinformatics, Duke University, Matteo Vestrucci, Berry Consultants, Ph.D, John Clark, Neurizon Therapeutics, B.Sc, Michael Thurn, Neurizon Therapeutics, Ph.D, Christian Freitag, Neurizon Therapeutics, MD

In collaboration with Berry Consultants, Neurizon will present new research exploring the use of external control (EC) cohorts to provide comparative context and improve interpretation of early-phase, single-arm ALS clinical trials, where concurrent randomised controls are typically not feasible. The study applies propensity-score-based statistical methodologies to leverage data from the PRO-ACT database—one of the largest ALS clinical trial repositories worldwide—to provide meaningful context for efficacy outcomes when randomized controls are not feasible.

Through simulation and a case study using data from the NUZ-001 Phase 1 trial and its open-label extension study, the research demonstrates that EC frameworks can strengthen early-stage decision-making by providing more rigorous, contextualized assessments of treatment outcomes such as survival and ALSFRS-R functional change.

This research exemplifies Neurizon's focus on innovative, data-driven clinical design and its commitment to advancing patient-centric, methodologically sound approaches that can accelerate therapeutic development in ALS.

Dr Michael Thurn, Managing Director and CEO, commented: "Being part of NEALS 2025 provides a unique opportunity to share our science and to collaborate with global experts who share our mission to transform outcomes for people living with ALS.

These two presentations highlight both the scientific depth behind NUZ-001 and the strength of our partnerships — combining advanced biology with innovative data science to better design, interpret, and accelerate ALS research. Together, we're working to move the field forward with purpose and precision and accelerating therapeutic innovation for people living with ALS and other neurodegenerative diseases."

The NEALS Annual Meeting brings together leading clinicians, researchers, patient advocates, and industry representatives from around the world to share the latest data and drive progress in ALS and motor neurone disease (MND) research.

For Neurizon, participation in this globally recognised forum represents an important opportunity to collaborate with the HEALEY ALS Platform Trial investigators, engage with key opinion leaders and researchers, and connect with patient organisations who share our mission to accelerate access to innovative, meaningful therapies for people living with ALS.

Copies of the abstracts and associated posters are available as attachments to this announcement.

-ENDS-

This announcement has been authorized for release by the Board of Neurizon Therapeutics Limited.
For further information, please contact:

Dr. Michael Thurn
Managing Director and Chief Executive Officer
Neurizon Therapeutics Limited
enquiries@neurizon.com
+61 (3) 9692 7222

Lidija Damjanovic
Head of Marketing and Corporate Affairs
Neurizon Therapeutics Limited
lidija@neurizon.com
+61 (0) 425 700 504

Released through: Henry Jordan, Six Degrees Investor Relations, +61 (0) 431 271 538

About Neurizon Therapeutics Limited

Neurizon Therapeutics Limited (ASX: NUZ) is a clinical-stage biotechnology company dedicated to advancing treatments for neurodegenerative diseases. Neurizon is developing its lead drug candidate, NUZ-001, for the treatment of ALS, which is the most common form of motor neurone disease. Neurizon's strategy is to accelerate access to effective ALS treatments for patients while exploring the potential of NUZ-001 for broader neurodegenerative applications. Through international collaborations and rigorous clinical programs, Neurizon is dedicated to creating new horizons for patients and families impacted by complex neural disorders. NUZ-001 is an investigational product and is not approved for commercial use in any jurisdiction.

Neurizon Investor Hub

We encourage you to utilise our Investor Hub for any enquiries regarding this announcement or other aspects concerning Neurizon.
This platform offers an opportunity to submit questions, share comments, and view video summaries of key announcements.

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Poster 1 Abstract: "Preclinical Characterization of NUZ-001: A Brain-Penetrant Small Molecule That Reduces TDP-43 Aggregation in ALS Models"

Background
Amyotrophic lateral sclerosis (ALS) is a progressive neurodegenerative disease characterized by cytoplasmic aggregation of TDP-43 in motor neurons. Despite advances in understanding ALS pathology, effective disease-modifying therapies remain limited. NUZ-001 is a novel small molecule therapeutic designed to target the pathological manifestations of disease pathology including TDP-43 aggregation.

Objective:
To evaluate the pharmacokinetic (PK) profile, brain penetration, and efficacy of NUZ-001 and its primary metabolite, NUZ-001 Sulfone, in reducing TDP-43 aggregation in preclinical ALS models.

Methods:
Pharmacokinetic studies were conducted in mice to quantify plasma and brain concentrations following systemic administration of NUZ-001. Compound levels were measured using validated LC-MS/MS assays. Brain levels of NUZ-001 and NUZ-001 Sulfone were compared against in vitro effective concentrations for TDP-43 aggregation reduction. To assess selectivity, both compounds were screened across multiple panels of CNS receptors and kinases at 10 µM to characterize overall pharmacology

Results:
In human IPSC-derived motor neurons carrying a TDP-43 mutation, M377V, both NUZ-001 and NUZ-001 Sulfone reduced pathological TDP-43 aggregation by up to 75% at brain-relevant concentrations. Following systemic dosing of NUZ-001, NUZ-001 and NUZ-001 Sulfone were readily detectable in brain tissue, achieving concentrations of 285-1,021 nM and 189-1314 nM respectively. Chronic dosing of NUZ-001 (10 days) showed no reduction in NUZ-001 or NUZ-001 Sulfone levels in plasma. Both compounds demonstrated high pharmacological selectivity, with no significant interaction (≥75% inhibition) across several tested targets. To evaluate to opportunity to expand therapeutic options for NUZ-001, a zebrafish model with reduced expression of the huntingtin gene was used to evaluate the protective effects of NUZ-001 and NUZ-001 Sulfone. Both NUZ-001 and NUZ-001 Sulfone reverse phenotypes and biochemical changes induced by HTT knockdown.

Conclusions/Next Steps:
These findings support on-going clinical trials for NUZ-001 in ALS and demonstrated brain penetration of both NUZ-001 and the active metabolite NUZ-001 Sulfone. Moreover, brain levels achieved in vivo exceed those required for reduction of TDP-43 aggregation in a human, iPSC-derived, ALS model. Studies evaluating the molecular targets of NUZ-001 and NUZ-001 Sulfone demonstrate a clean receptor pharmacology. Ongoing studies are evaluating the molecular mechanisms underlying TDP-43 reduction by NUZ-001 and NUZ-001 Sulfone and the potential to expand therapeutic options for NUZ-001 in other neurodegenerative disease.

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Poster 2 Abstract: "Leveraging External Controls from PRO-ACT for Exploratory Efficacy Assessment in Early-Phase ALS Trials"

Background
Amyotrophic lateral sclerosis (ALS) is a progressive and fatal neurodegenerative disease for which effective treatments remain limited, highlighting a high unmet medical need. Early-phase clinical trials, particularly Phase 1 studies, are critical for assessing safety and pharmacokinetics of investigational therapies. However, in the context of ALS, these studies are increasingly relied upon to generate preliminary efficacy signals to inform go/no-go decisions for further development and to secure ongoing investment. These trials are commonly single-arm and open-label with long follow-up periods, which presents challenges in interpreting exploratory outcomes due to the absence of a concurrent control group.

To address this limitation, external controls (ECs) derived from historical clinical trial data offer a promising strategy to contextualize efficacy outcomes in early-phase ALS studies. The Pooled Resource Open-Access ALS Clinical Trials (PRO-ACT) database is one of the largest publicly available sources of participant-level data from over 30 randomized Phase 2/3 ALS trials. The database is regularly updated with additional high-quality datasets from recent, well-conducted trials, further enhancing its value as a comparator resource. PRO-ACT offers a robust and well-curated dataset for constructing ECs in exploratory analyses.

However, the use of ECs is methodologically complex due to potential heterogeneity between trial participants and external data sources. To account for this, propensity score (PS)-based methods are commonly employed to adjust for observed confounding and improve comparability.

Methods and Results
We evaluated through simulation the application of PS-based approaches to create ECs from the PRO-ACT database and compared their performance to hypothetical randomized designs in early-phase ALS trials. Key metrics included balance in baseline covariates, treatment effect estimates, and robustness to model specifications. As a case study, we applied these methods to data from a Phase 1 open-label trial of NUZ-001, assessing exploratory efficacy endpoints including survival and change in ALS Functional Rating Scale-Revised (ALSFRS-R). After either matching or weighting using PS techniques, ECs from PRO-ACT were compared with trial participants for treatment effect estimation, and comparative analyses were conducted.

Results demonstrate that PS-based methods can achieve acceptable covariate balance and provide interpretable comparative estimates of treatment effect. In the NUZ-001 case study, exploratory analyses suggested a potential therapeutic benefit on survival and ALSFRS-R decline relative to matched PRO-ACT controls, supporting the value of ECs in early-phase decision-making.

Preclinical Characterization of NUZ-001: A Brain-Penetrant Small Molecule That Reduces TDP-43 Aggregation in ALS Models

Poster 153

Jeffrey M. Brown, PhD, MBA and Michael Thurn, PhD. Neurizon Therapeutics, Suite 2, Level 11, 385 Bourke Street, Melbourne, VIC 3000, Australia

BACKGROUND

Amyotrophic lateral sclerosis (ALS) is a progressive neurodegenerative disease characterized by cytoplasmic aggregation of TDP-43 in motor neurons. Despite advances in understanding ALS pathology, effective disease-modifying therapies remain limited. NUZ-001 is a novel small molecule therapeutic designed to target the pathological manifestations of disease pathology including TDP-43 aggregation.

OBJECTIVE

To evaluate the pharmacokinetic (PK) profile, brain penetration, and efficacy of NUZ-001 and its primary metabolite, NUZ-001 Sulfone, in reducing TDP-43 aggregation in preclinical ALS models.

METHODS

Pharmacokinetic studies were conducted in mice to quantify plasma and brain concentrations following systemic administration of NUZ-001. Compound levels were measured using validated LC-MS/MS assays. Brain levels of NUZ-001 and NUZ-001 Sulfone were compared against in vitro effective concentrations for TDP-43 aggregation reduction. To assess selectivity, both compounds were screened across multiple panels of CNS receptors and kinases at 10 µM to characterize overall pharmacology

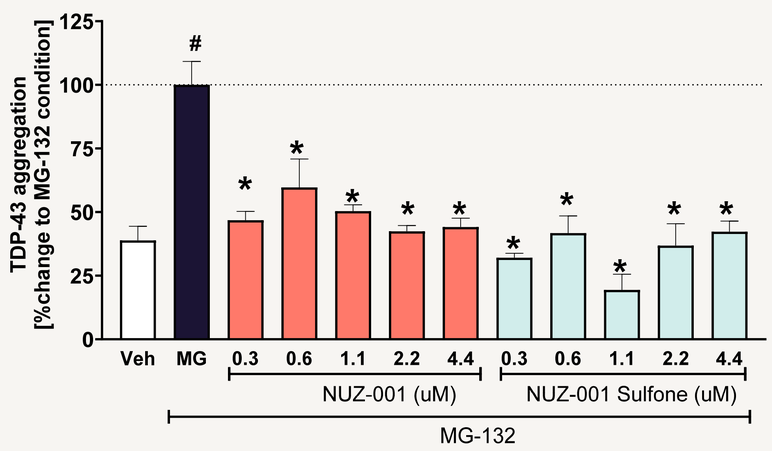
RESULTS

In human IPSC-derived motor neurons carrying a TDP-43 mutation, M377V, both NUZ-001 and NUZ-001 Sulfone reduced pathological TDP-43 aggregation by up to 75% at brain-relevant concentrations. Following systemic dosing of NUZ-001, NUZ-001 and NUZ-001 Sulfone were readily detectable in brain tissue, achieving concentrations of 285-1,021 nM and 189-1314 nM respectively. Chronic dosing of NUZ-001 (10 days) showed no reduction in NUZ-001 or NUZ-001 Sulfone levels in plasma. Both compounds demonstrated high pharmacological selectivity, with no significant interaction (≥75% inhibition) across several tested targets. To evaluate to opportunity to expand therapeutic options for NUZ-001, a zebrafish model with reduced expression of the huntingtin gene was used to evaluate the protective effects of NUZ-001 and NUZ-001 Sulfone. Both NUZ-001 and NUZ-001 Sulfone reverse phenotypes and biochemical changes induced by HTT knockdown.

CONCLUSION / NEXT STEPS

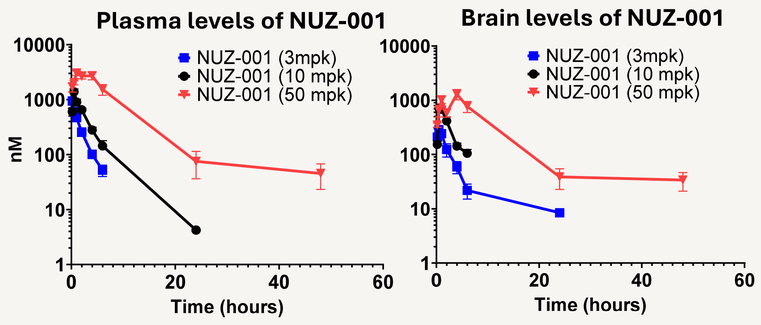
These findings support on-going clinical trials for NUZ-001 in ALS and demonstrated brain penetration of both NUZ-001 and the active metabolite NUZ-001 Sulfone. Moreover, brain levels achieved in vivo exceed those required for reduction of TDP-43 aggregation in a human, iPSC-derived, ALS model. Studies evaluating the molecular targets of NUZ-001 and NUZ-001 Sulfone demonstrate a clean receptor pharmacology. Ongoing studies are evaluating the molecular mechanisms underlying TDP-43 reduction by NUZ-001 and NUZ-001 Sulfone and the potential to expand therapeutic options for NUZ-001 in other neurodegenerative disease.

FIGURE 1: NUZ-001 AND NUZ-001 SULFONE REDUCED TDP-43 AGGREGATION IN A TDP-43 MUTANT IPSC-DERIVED MOTOR NEURON MODEL



Human iPSC-derived motor neurons with the TDP-43 M337V mutation were treated with MG-132 (0.2 µM) for seven days to induce protein aggregation. NUZ-001 or NUZ-001 Sulfone was co-administered at increasing concentrations. Efficacy was measured against the MG-132 alone group (∗ P<0.05 vs MG132; # P<0.05 vs vehicle).

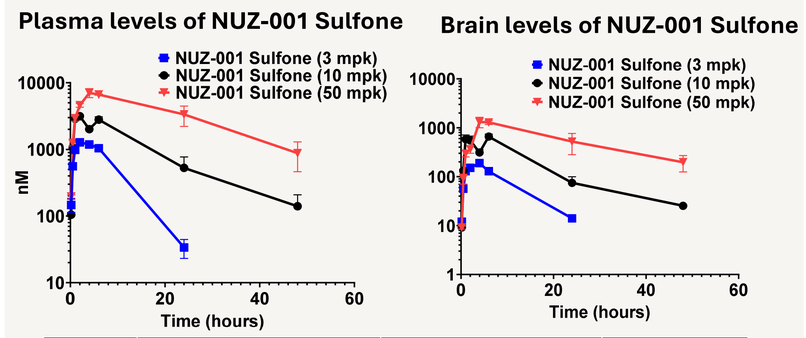
FIGURE 2: NUZ-001 ACHIEVES µM EXPOSURE IN BRAIN AND PLASMA



Dose	NUZ-001 plasma (nM)	NUZ-001 Brain (nM)	B/P ratio
3	947	285	0.3
10	1386	708	0.5
50	3048	1021	0.3

Male ICR mice were dosed with a single administrating of NUZ-001 @ 3, 10, 50 mpk. Plasma and brain samples were collected at multiple time points and NUZ-001 quantified by LC-MS/MS.

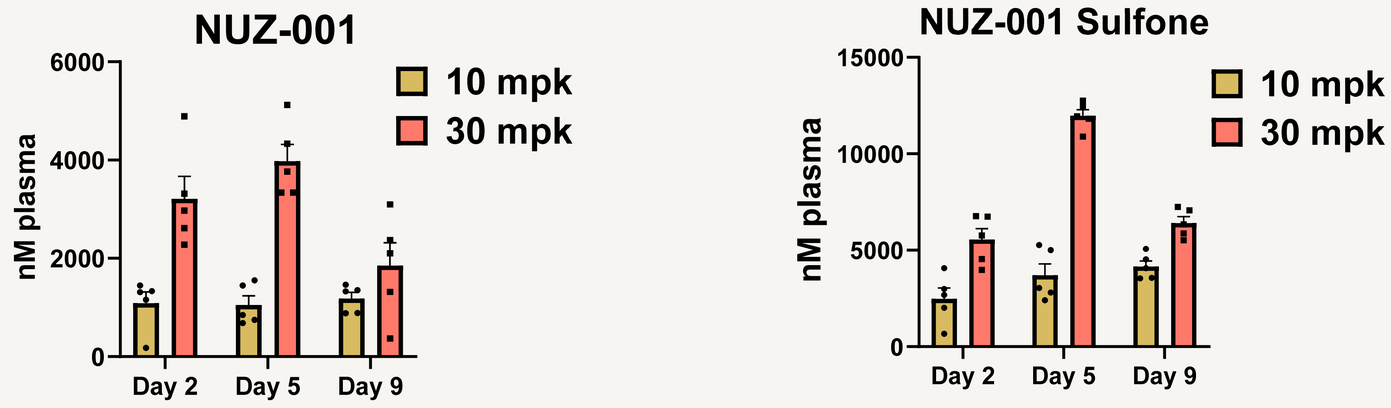
FIGURE 3: NUZ-001 SULFONE SHOWS PROLONGED BRAIN EXPOSURE FOLLOWING NUZ-001 ADMINISTRATION



Dose (mpk)	NUZ-001 Sulfone plasma (nM)	NUZ-001 Sulfone Brain (nM)	B/P ratio
3	1274	189	0.2
10	3142	582	0.2
50	7707	1314	0.2

Male ICR mice were administered a single administrating of NUZ-001 @ 3, 10, 50 mpk. Plasma and brain samples were collected at multiple time points and NUZ-001 Sulfone quantified by LC-MS/MS.

FIGURE 4: MULTIPLE DOSES OF NUZ-001 RESULTS IN SUSTAINED PLASMA LEVELS OF NUZ-001 AND NUZ-001 SULFONE



Dose	NUZ-001 concentration @ 2hrs					
	Day 2		Day 5		Day 9	
mpk	Plasma (nM)	Est Brain (nM)	Plasma (nM)	Est Brain (nM)	Plasma (nM)	Est Brain (nM)
10	1085	434	1055	422	1182	473
30	3215	1250	3979	1591	1852	741

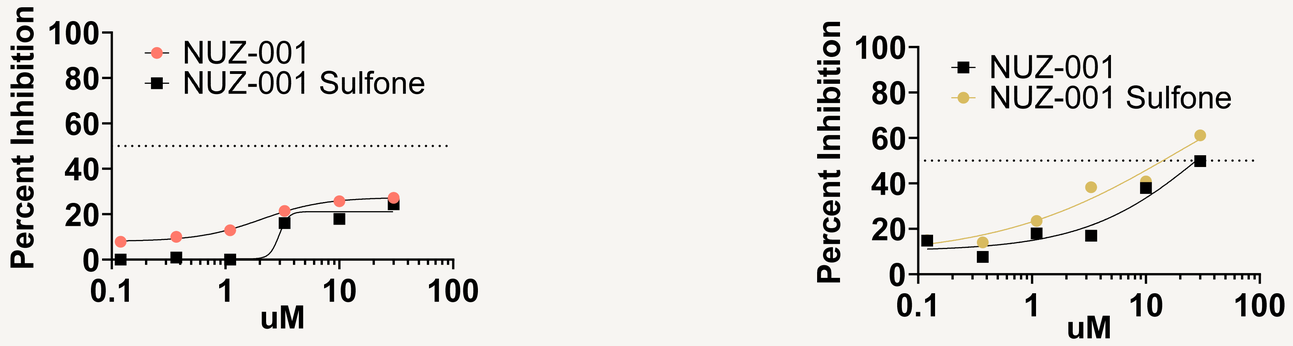
Dose	NUZ-001 Sulfone concentration @ 2hrs					
	Day 2		Day 5		Day 9	
mpk	Plasma (nM)	Est Brain (nM)	Plasma (nM)	Est Brain (nM)	Plasma (nM)	Est Brain (nM)
10	2485	497	3704	741	4154	831
30	5557	1111	11968	2393	6704	1340

Male C57 mice were dosed with NUZ-001 @ 10 mpk or 30 mpk once per day for 10 days. Plasma samples were collected 2 hrs post administration of NUZ-001. NUZ-001 and NUZ-001 sulfone were quantified by LC-MS/MS. Brain levels were estimated based on a B/P ratio of 0.4 and 0.2 for NUZ-001 and NUZ-001 Sulfone respectively.

FIGURE 5: NUZ-001 AND NUZ-001 SULFONE SHOW CLEAN PHARMACOLOGY AT MULTIPLE PHARMACOLOGICAL TARGETS

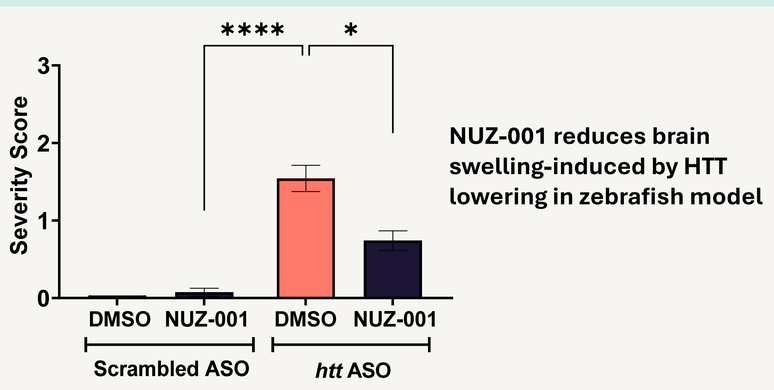
NUZ-001 and NUZ-001 Sulfone do not inhibit α7 Nicotinic receptors

NUZ-001 and NUZ-001 Sulfone do not inhibit calcium channels

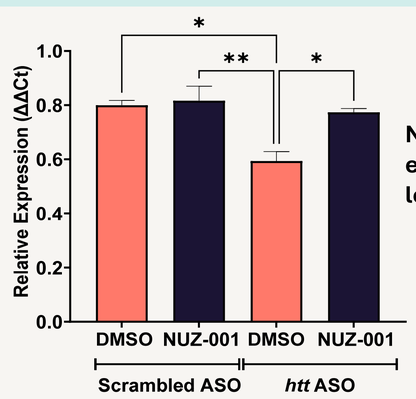


In receptor binding, functional orphan GPCRs, and kinase assays, NUZ-001 and NUZ-001 Sulfone showed <75% activity @ 10uM at all targets tested. Minor effects were seen at α7 nicotinic receptors and L-type calcium channels. Follow-up functional studies show IC50s ≥20uM at α7 and CaV1.2, well above plasma and brain levels achieved in vivo and in clinical testing (see figure 4)

FIGURE 6: NEUROPROTECTIVE EFFECTS OF NUZ-001 AND NUZ-001 SULFONE EXTEND TO HUNTINGTON DISEASE MODELS



NUZ-001 reduces brain swelling-induced by HTT lowering in zebrafish model



NUZ-001 restores BDNF expression following HTT lowering in zebrafish model

Expression of the huntingtin gene (*htt*) was reduced in Zebrafish using and antisense oligonucleotide (ASO). NUZ-001 and NUZ-001 Sulfone (data not shown) restored the phenotypic and biochemical deficits induced by *Htt* lowering.

CONCLUSION: NUZ-001 and its metabolite, NUZ-001 Sulfone, effectively prevented TDP-43 aggregation in human iPSC-derived motor neurons. The observed efficacy at nanomolar (nM) concentrations is highly relevant, as this range is readily achievable in the brain according to mouse and human pharmacokinetic data. This establishes NUZ-001 as a potent, brain-penetrant lead candidate for ALS. Furthermore, preliminary data suggest NUZ-001's beneficial effects may expand beyond ALS to other neurodegenerative diseases, including Huntington's Disease.

Leveraging External Controls from PRO-ACT for Exploratory Efficacy Assessment in Early-Phase ALS Trials

BERRY

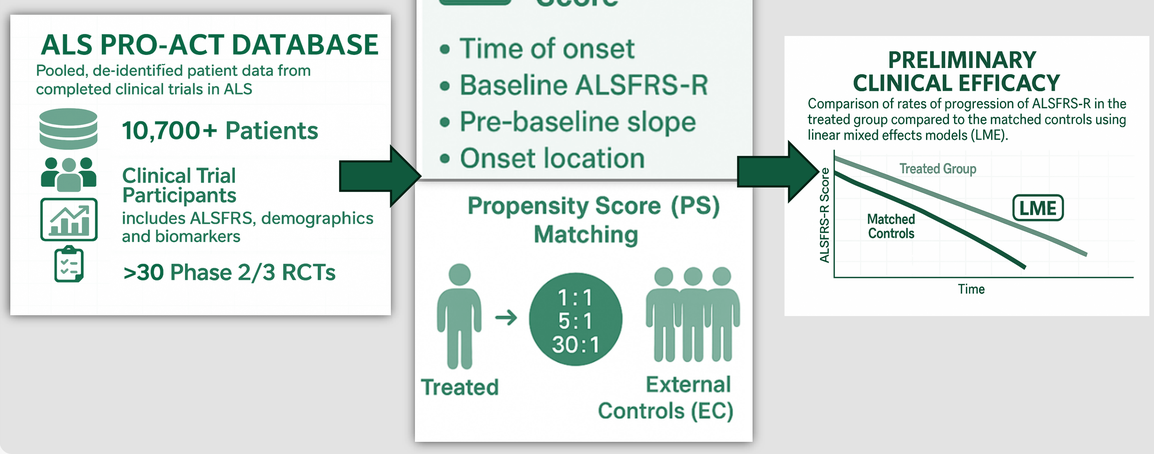
Neurizon

Demonstrating of the feasibility of using external controls derived from the PRO-ACT database to facilitate preliminary efficacy assessment in early-phase, single-arm ALS trials. Through a case example with NUZ-001 and a simulation study, EC methods achieved covariate balance and yielded interpretable treatment effect estimates, supporting their role in strengthening early clinical development.

Background

- **ALS is a progressive, fatal neurodegenerative disease** with limited treatment options, highlighting a high unmet need.
- **Early-phase trials in ALS (especially Phase 1)** are designed for safety and pharmacokinetic evaluation but are increasingly expected to provide **preliminary efficacy signals.**
- These trials are often **single-arm and open-label**, making efficacy interpretation challenging without concurrent controls.
- **External controls (ECs)** using historical trial data provide a potential solution to improve comparative context.
- The **PRO-ACT database** contains participant-level data from >30 randomized Phase 2/3 ALS trials, serving as a valuable source for EC construction.
- **Propensity score (PS) methods** can adjust for population differences, improving comparability and reducing bias in EC analyses.

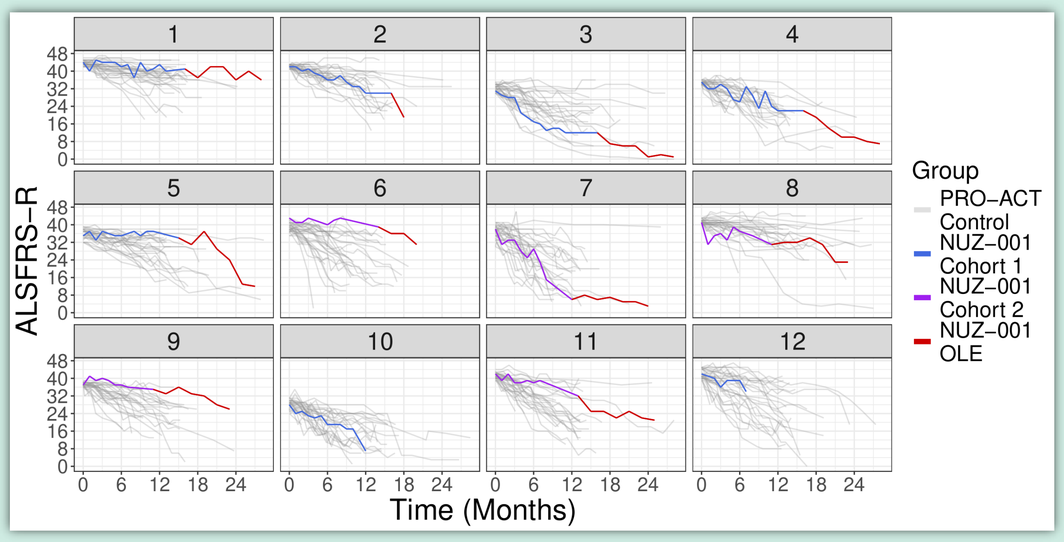
Matching procedure + Preliminary Efficacy Analysis



Motivating Example: NUZ-001 Integrated Preliminary Efficacy Analysis

- N=12 NUZ-001 treated participants from Phase 1 + OLE compared to PS matched EC from PRO-ACT
- Baseline covariates are well balanced after matching

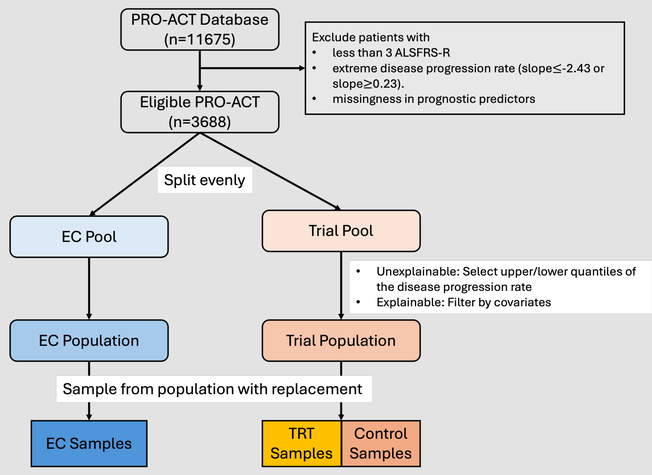
	PRO-ACT Controls (N = 360)	NUZ-001 (N = 12)
Time since Onset (Months)		
Mean (SD)	15.3 (7.27)	14.7 (8.38)
Median [Min,Max]	14.5 [3.8, 35.8]	13.9 [3.6, 34.0]
Baseline ALSFRS-R		
Mean (SD)	37.9 (4.64)	38.2 (5.10)
Median [Min,Max]	38.0 [24.0, 46.0]	39.5 [28.0, 44.0]
Pre-Baseline Slope		
Mean (SD)	0.786 (0.438)	0.826 (0.461)
Median [Min,Max]	0.733 [0.11, 2.14]	0.739 [0.21, 1.48]
Onset Location		
Bulbar	60 (16.7%)	2 (16.7%)
Other	300 (83.3%)	10 (83.3%)



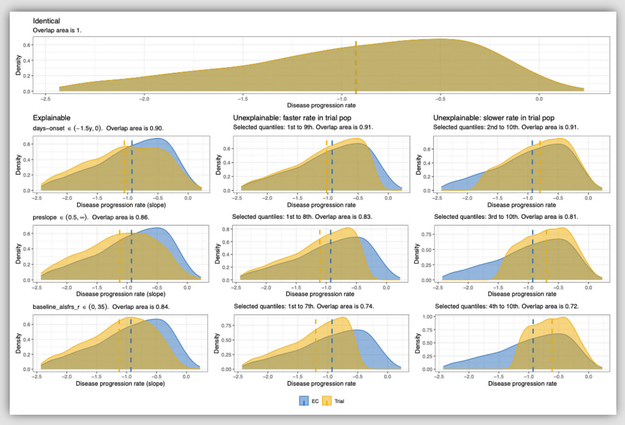
Matching analysis	PRO-ACT Control Slope	NUZ-001 Slope (N=12)	Between-group Difference	P-value (one-sided)
30:1	-1.21 (-1.30, -1.11)	-0.83 (-1.33, -0.34)	0.37 (-0.13, 0.88)	0.0725
5:1	-1.28 (-1.49, -1.06)	-0.83 (-1.33, -0.34)	0.44 (-0.05, 0.94)	0.0380
1:1	-1.26 (-1.66, -0.86)	-0.83 (-1.33, -0.34)	0.43 (-0.13, 0.99)	0.0645

Simulation Study using PRO-ACT

PRO-ACT database is split evenly into trial population and EC population.



Trial population is selected to mimic scenarios with explainable (by covariates) or unexplainable differences in the rates of progression on ALSFRS-R compared to EC.



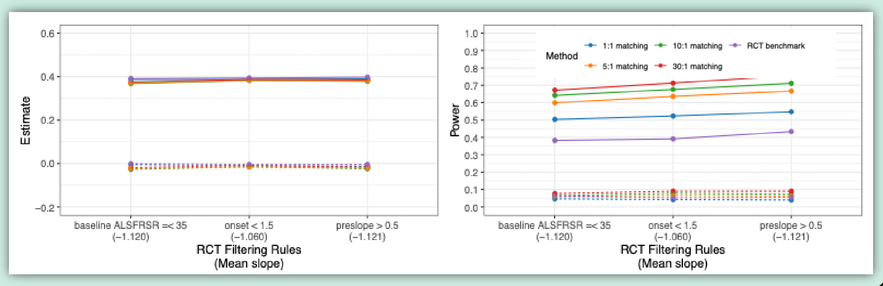
- Single arm analyses with different matching ratios are compared to RCT and naïve historical benchmark
- N=12 for single arm trial vs. N=24 (1:1 ratio) in RCT
- Treatment effects include no difference in slope (Null) and a difference in the slope of 0.40
- Overall average operating characteristics, effect estimates and power/type I error under one-sided alpha of .05, are reported

Simulation Comparison of Single Arm + EC to RCT for Small Phase 1 studies (N=12)

Matching results in unbiased estimates.

Power using matching can be increased over RCT in studies with small N. Likely due to potential for cov. Imbalance.

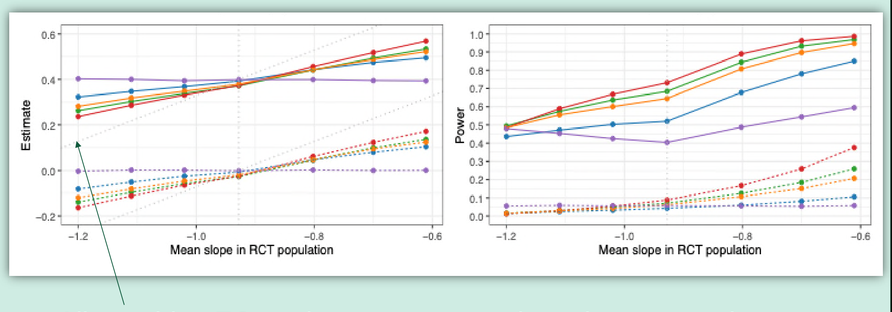
Explainable Differences between EC and Trial Population



Matching EC analyses result in bias estimates and inflation in Type I Error

- *Likely small under 1:1 matching. Effect estimates range from 0.31 to 0.50. Type I error increases from 5% to 10%.*
- *Increases with matched ratio.* *Note: May not translate to what is often done in practice where matching ratio is chosen dynamically*

Unexplainable Differences between EC and Trial Population



All matching EC analyses are more robust than comparing to a niave historical benchmark with reductions in total potential bias of up to 67%.

Conclusions

External controls (ECs) from PRO-ACT can facilitate preliminary efficacy assessments in early-phase, single-arm ALS trials

Strengths

- PRO-ACT is a rigorously collected clinical trial database where endpoints are measured consistently with clinical trial standards, reducing potential measurement bias.
- Large pool of EC enables robust matching on key prognostic covariates.
- Known prognostic factors in ALS progression are well studied, supporting reliable adjustment methods.
- PS methods provide robust results if differences in EC and trial population are explained by covariates.
- Under the assumption of unmeasured confounders, formal matching analyses are more robust than naïve historical benchmark comparisons

Limitations

- Potential unmeasured confounders could result in unexplained differences between EC and trial data.
- Unmeasured confounders may include regional differences or differences between populations likely to enter Ph1 vs. Ph 2/3 trials.
- In the presence of unmeasured differences, PS matched analyses can result in biased estimates of treatment effects and inflated type I error.
- Quantifying the potential degree of bias and type I error inflation is important. Treatment effect estimates should be considered in light of this.
- Future work is needed to explore optimal EC methods and prognostic baseline covariates

Authors

Melanie Quintana[1], Philip White[2], Peijin Wang[3], Matteo Vestrucci[1], John Clark[4], Michael Thurn[4], Christian Freitag[4]

[1]Berry Consultants; [2]Department of Statistics, Brigham Young University; [3]Department of Biostatistics and Bioinformatics, Duke University; [4]Neurizon Therapeutics

Acknowledgements

The authors would like to thank all the participants and their families, Site Staff, and Study Partners who participated in the NUZ-001 Phase 1 study and OLE.

Data used for simulation were obtained from the Pooled Resource Open-Access ALS Clinical Trials (PRO-ACT) database.