Neuren presents at Therapeutic Area Partnerships Conference; announces positive results in Fragile X Syndrome model

SYDNEY, Australia, 30 November 2012: Neuren Pharmaceutical Limited (ASX:NEU) presented at the Therapeutic Area Partnerships conference in Boston, Massachusetts today. The conference was sponsored by Windhover, a subsidiary of Elsevier Business Communications. Dr. Joe Horrigan, Neuren's Vice President of Clinical Development and Medical Affairs, provided an overview and update on the Company's NNZ-2566 program. A copy of the presentation is attached and will be posted on Neuren's website www.neurenpharma.com. As previously announced on 19th October this year, the NNZ-2566 program was selected by Windhover's expert panel as one of the top 10 neuroscience projects for 2012.

During the presentation, Neuren also announced positive results with NNZ-2566 in a recently completed mouse model of Fragile X Syndrome. Fragile X Syndrome is the most common form of inherited intellectual disability and has many overlapping features with autism spectrum disorders. In this study, beneficial changes were evident on both anatomic and behavioural symptoms of the mice.

Commenting on the results of this study, Dr. Horrigan said, "We are very excited. The positive changes in behavior and anatomy that we saw in the test animals were very clear, at targeted exposures that we believe are similar to what might be therapeutic for patients.

How well these improvements could translate into what patients might experience is unknown at present. However, when these new findings are viewed together with our existing preclinical data, they build confidence in the potential for NNZ-2566 to be a valuable medicine in the treatment of neurodevelopmental disorders such as Rett and Fragile X syndromes. This is important for patients and families because, at the current time, consistently helpful medicines are lacking for individuals affected by these conditions. NNZ-2566 represents a new and distinct therapeutic approach in this field.

Also, it is possible that benefit in treating these less common disorders may predict benefit in treating the more common types of autism, in light of the similar pathophysiology. But that concept will need to be formally tested".

Neuren has completed five Phase I safety and pharmacokinetics studies with NNZ-2566 in human volunteers, four with the intravenous solution and one with the oral solution. NNZ-2566 appeared to be safe and well-tolerated in healthy volunteers. The IND for a Phase II study of NNZ-2566 in adolescent and adult subjects with Rett Syndrome was recently filed, and the Company is considering additional clinical trials in other autism spectrum or neurodevelopmental disorders.

About NNZ-2566

NNZ-2566 is a synthetic analogue of a naturally occurring neuroprotective and neurotrophic molecule derived from IGF-1, a growth factor produced by brain cells as well as in other parts of the body. The intravenous form of NNZ-2566 is presently in a Phase II clinical trial in patients with moderate to severe traumatic brain injury which has received Fast Track designation from the US FDA. The company is currently undertaking final preparations to initiate two additional Phase II trials with the oral form of NNZ-2566 – one in patients with concussion or mild TBI and one in patients with Rett Syndrome.

About Rett Syndrome

Rett Syndrome is a post-natal neurological disorder which occurs almost exclusively in females following apparently normal development for the first six months of life. Typically, between 6 to 18 months of age, patients experience a period of rapid decline with loss of purposeful hand use and spoken communication. Many patients have recurrent seizures. They experience a variety of motor problems including increased muscle tone (spasticity) and abnormal movements. They are never able to provide for their own needs. It is a rare disorder and is believed to be second only to Down Syndrome as a cause of chronic neurological problems that include severe communication, motor disabilities and epilepsy. Rett Syndrome is caused by mutations on the X chromosome of a gene called MECP2. There are more than 200 different mutations found on the MECP2 gene. Rett Syndrome strikes all racial and ethnic groups, and occurs worldwide in up to 1 of every 10,000 female births and affects some 15,000 girls and women in the U.S. alone.

About Fragile X Syndrome

Fragile X syndrome is the most common inherited cause of intellectual disability, and the most common known cause of autism. It affects 1 out of 4000 males and 1 out of 6-8000 females. Fragile X syndrome is due to a single gene defect on the X chromosome that impacts the FMRP protein, which is responsible for regulating the synapses of nerve cells. Clinically, Fragile X Syndrome is characterized by intellectual handicap, hyperactivity and attentional problems, autistic symptoms, anxiety, emotional lability and epilepsy. The epilepsy seen in Fragile X Syndrome is most commonly present in childhood, but then gradually improves towards adulthood. Physical features such as prominent ears and jaw, and hyper-extensibility of joints are frequently present but are not diagnostic. Generally, males are more severely affected than females. Currently, there are no medicines approved for the treatment of Fragile X Syndrome.

About Neuren

Neuren Pharmaceuticals is a biopharmaceutical company developing new therapies for brain injury, neurodevelopmental and neurodegenerative disorders and cancer. Neuren presently has two clinical-stage molecules, NNZ-2566 and Motiva®, in Phase 2 clinical trials largely funded by the US Army and the National Health and Medical Research Council, respectively. Through its subsidiary, Perseis Therapeutics Limited, Neuren is developing monoclonal antibodies against Trefoil Factors 1 and 3, proteins produced by cancer cells that are associated with cancer spread and reduced patient survival.

For more information, please contact:

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Therapeutic Area Partnerships
November 2012

Forward Looking Statement

This presentation contains forward looking statements that involve risks and uncertainties. Although we believe that the expectations reflected in the forward looking statements are reasonable at this time, Neuren can give no assurance that these expectations will prove to be correct.

Actual results could differ materially from those anticipated. Reasons may include risks associated with drug development and manufacture, risks inherent in the regulatory processes, delays in clinical trials, risks associated with patent protection, future capital needs or other general risks or factors.

Company Overview

Larry Glass, Chief Executive Officer

30+ years of experience in management and corporate development of life sciences companies; former CEO of CRO supporting pharmaceutical and biotechnology companies and US government agencies including NIH, CDC and the US Army; 9 years with Neuren

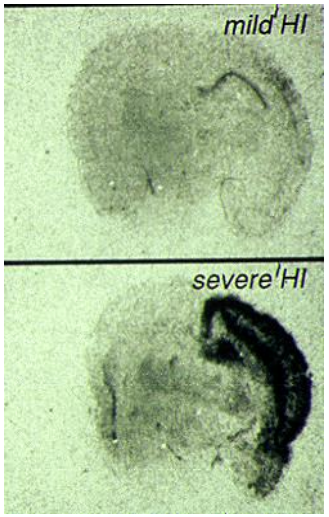
Joe Horrigan, MD, VP, Clinical Development & Medical Affairs

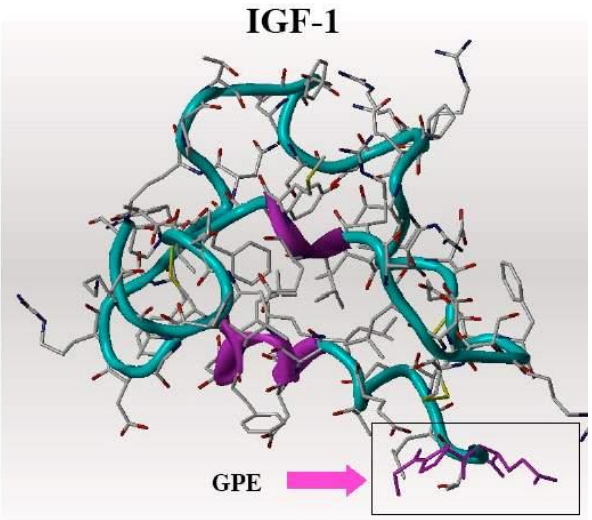
20+ years in academic medicine and drug development; former Asst. VP, Head of Medical Research, Autism Speaks; former Executive Director in Neurosciences Medicines Development Center and Director, Medicines for Children Advisory Network, GlaxoSmithKline

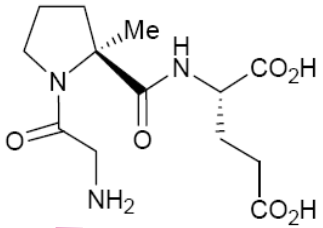
- New Zealand company; spun out from University of Auckland
- Listed on Australian Securities Exchange (ASX:NEU)
- Operations
  - CEO/Managing Director and VP, Clinical Development & Medical Affairs in the US
  - Finance, clinical operations, preclinical R&D, quality assurance and regulatory affairs in NZ
- Key relationships
  - US Army Medical Research and Materiel Command/Walter Reed Army Institute of Research
  - International Rett Syndrome Foundation
  - Autism Therapeutics Ltd.
  - Baylor College of Medicine/Texas Children's Hospital
  - AMRI (Albany Molecular Research, Inc.)
  - Aptuit Ltd.

NNZ-2566

- IGF-1 is essential for brain development, widely expressed in the CNS, upregulated following brain injury
- Naturally occurring neurotrophic factor – one of the brain's self-repair mechanisms
- (1-3)IGF-1 or GPE is believed to be an active neuroprotective moiety in response to injury
- (1-3)IGF-1 appears to upregulate IGF-1 in the brain, enhance IGF1R phosphorylation and expression of synaptic markers
- NNZ-2566 is a (1-3)IGF-1 analog
  - Longer half-life
  - Crosses blood brain barrier
  - Orally available (45-50%)
  - Conventional solution phase synthesis
  - Validated manufacturing process
  - Excellent stability
- Strong patent estate
  - 7 issued patents
  - 5 pending applications

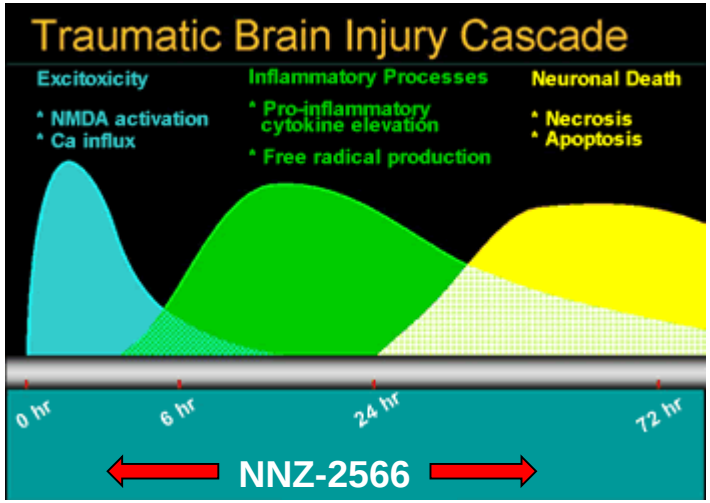


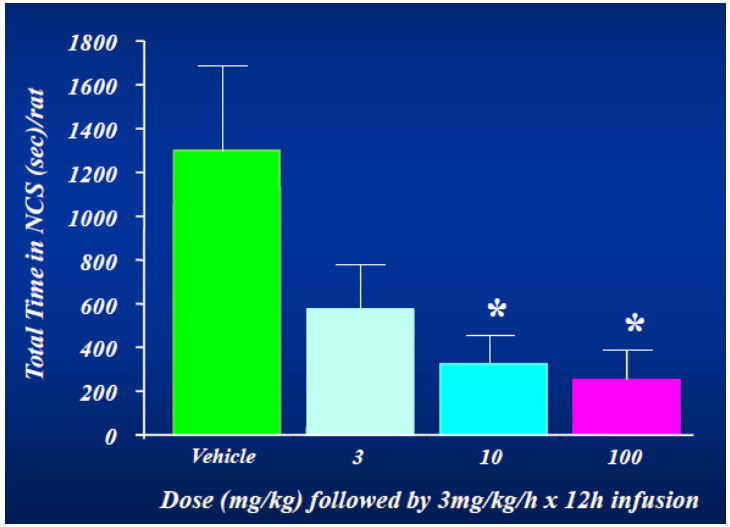




NNZ-2566

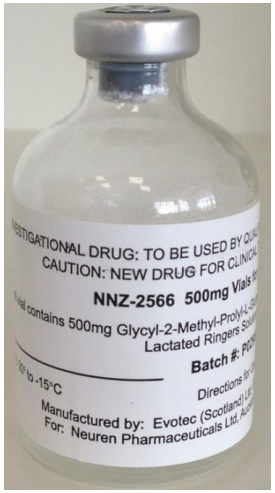
- Multiple mechanisms of action
  - Inhibits neuroinflammatory cytokine expression
  - Inhibits pathologic activation of microglia
  - Normalizes pro- and anti-apoptotic gene and protein expression
  - Enhances synaptic plasticity
  - Induces neuroblast proliferation
  - Inhibits post-injury seizures
- Effects in multiple animal models
  - Cortical concussive brain injury
  - Penetrating brain injury
  - Non-convulsive seizures
  - Stroke (MCAO with endothelin and ligation)
  - Aged rat
  - Neonatal hypoxia – ischemia
  - Rett Syndrome
  - Fragile X Syndrome

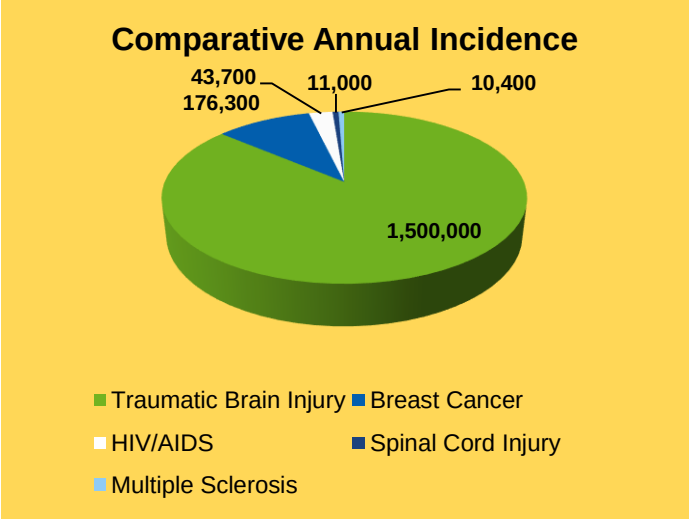




NNZ-2566 (intravenous formulation)

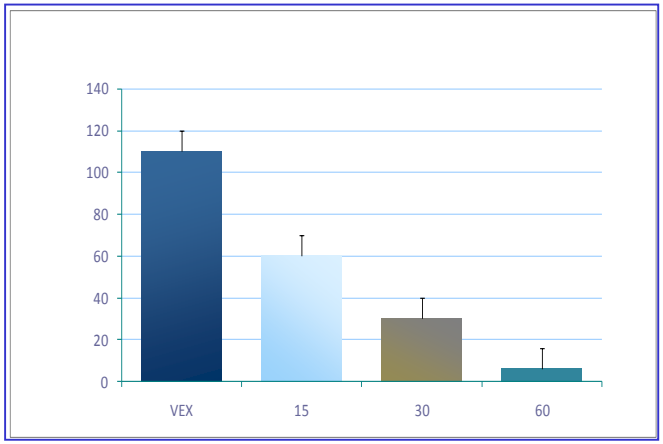
- Well-tolerated with no SAEs in 4 Phase I clinical trials
- *INTREPID*$^{-2566}$ trial underway
  - Randomized, double-blind, dose-escalation Phase II clinical trial
  - Fast Track designation granted by FDA
  - Exception from Informed Consent (EFIC) granted by FDA
  - >1/3 of subjects recruited
  - Appears to be safe and well-tolerated in TBI patients; DSMC has reviewed safety data on 80 patients at 3 doses; enrollment continued
  - Clinical assessments
    - Safety
    - Pharmacokinetics
    - Global outcomes
      - Glasgow Outcome Scale Extended
      - Mayo Portland Adaptability Inventory
    - Epileptiform discharges (detected by continuous EEG)
    - Neuropsychological assessments
    - Post-traumatic amnesia
    - Biomarkers (UCH-L1, GFAP, SDBP145)





NNZ-2566 (oral formulation)

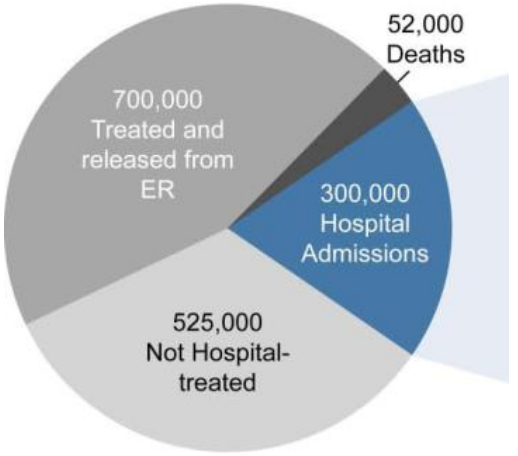
- Readily absorbed from the gut; crosses blood-brain barrier
- Targets: inflammation, synaptic function, and microglial activation
- Dose-dependent effects in animal models with delayed administration
- No clinical toxicity in rats at highest dose tested for 28 days
- No SAEs, no clinically significant AEs in Phase I trial up to 100 mg/kg b.i.d. x 5 days
- Formulated with water
  - Peak plasma level at 2 hours with or without food
  - Uses same drug substance as IV formulation – flexibility and cost-effectiveness
- Creates opportunities for chronic dosing and treating less severe injuries



Infarct volume after oral administration 3 hrs post-injury

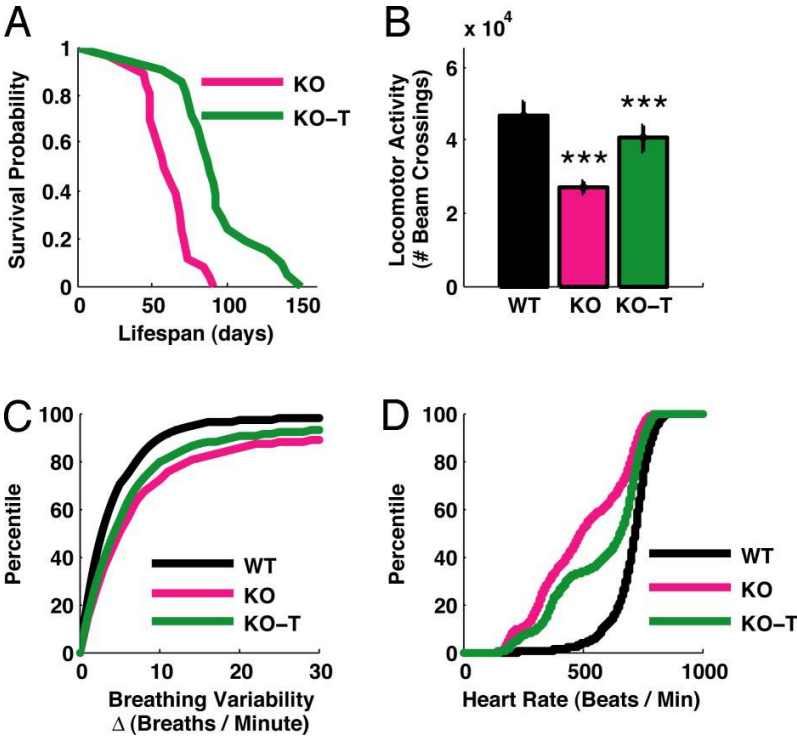
NNZ-2566 (oral): concussion

- IND open
- IGF-1 is upregulated in mild head injury
- Inflammation, microglial activation are prominent
- Phase II clinical trial preparations underway
  - 132 subjects; 2 doses plus placebo; 28 day follow-up
  - Subjects will have baseline neurocognitive assessment with ImPACT test
  - Comparison to pre-injury cognitive function expected to be more sensitive
  - Additional endpoints: balance, mood and global function
- Large market opportunity
  - Significant unmet need; major problem in athletes, military, young adults and aging populations
  - 700,000 treated in the emergency department, >500,000 not hospital treated
  - Post-concussion symptoms can last for months
  - Multiple concussions can result in severe, long-term disability



NNZ-2566 (oral): Rett Syndrome

- Neurodevelopmental disorder caused by mutation in MeCP2 gene
- Synaptic dysfunction, microglial activation, neuroinflammation appear to play prominent roles in Rett symptomatology
- Positive effects of IGF-1, (1-3)IGF-1 and NNZ-2566 observed in Rett syndrome models
- IGF-1 rescues glutamate receptor deficit in Rett neurons
- Wild-type microglia rescue phenotype in MeCP2 KO mice
- Two Phase I trials of rhIGF-1* have shown effects on symptoms



Tropea et al. Partial reversal of Rett Syndrome-like symptoms in MeCP2 mutant mice. PNAS, 2009.

* Increlex® (mecasermin); Ipsen Biopharmaceuticals

NNZ-2566 (oral): Rett Syndrome

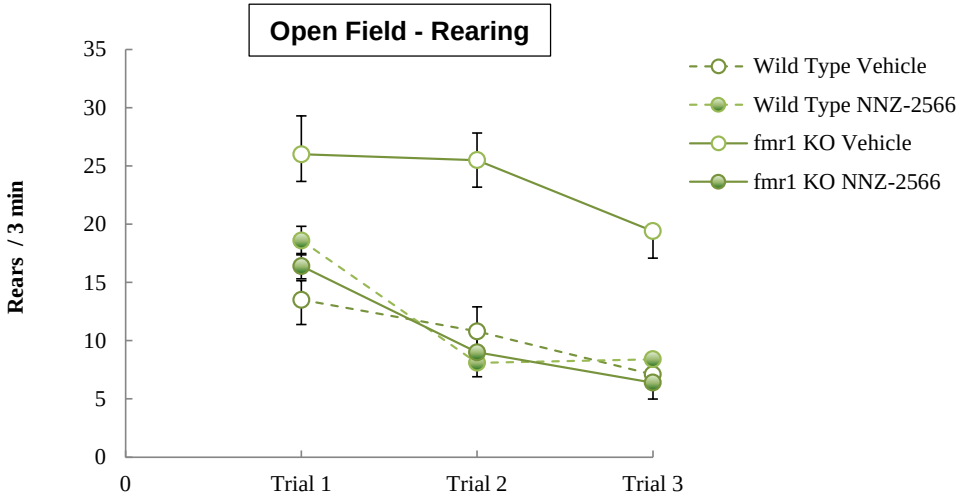
- Phase II trial of NNZ-2566 in adolescent and adult patients scheduled to open in December 2012
  - IND submitted in November following receipt of final Phase I study report
  - 48 subjects 16-40 years; two dose cohorts; randomized 2:1; stratified by mutation
  - 28 day treatment: dose escalation days 1-5, fixed dose days 6-23, taper days 23-28
  - Assessments: safety, PK, EEG, cardiac and respiratory function, behavior, global function
  - Lead site: Blue Bird Circle Rett Center (Baylor College of Medicine/Texas Children's)
  - Lead investigators: Daniel Glaze, MD; Jeffrey Neul, MD, PhD
  - Partially funded by International Rett Syndrome Foundation grant to Baylor
  - Targeted enrolment period = 12 months
  - Planning to include a second clinical site in Q1 2013
- Phase II trial in pediatric Rett Syndrome patients planned for 2013
- IND expected to support potential trials in other neurodevelopmental and autism spectrum disorders

NNZ-2566: Fragile X Syndrome study

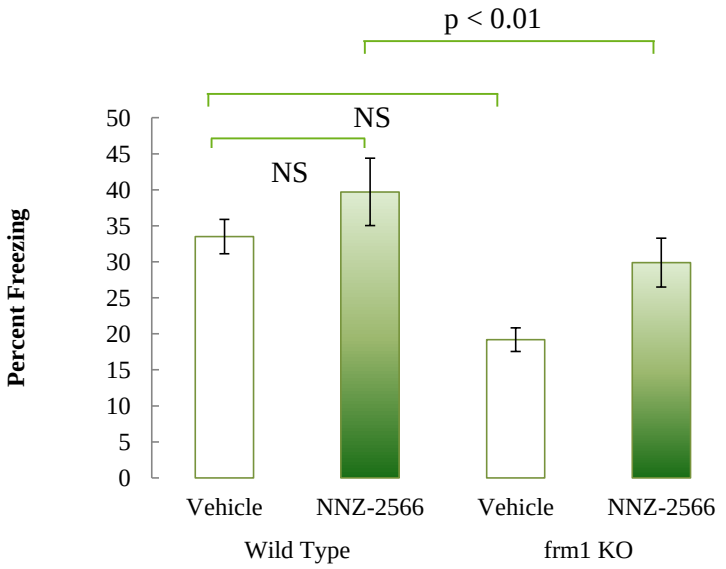
- The effect of NNZ-2566 was investigated in Wild Type mice and the *fmr1* KO mouse model of Fragile X Syndrome
- 100 mg/kg i.p. NNZ-2566 or vehicle administered for 28 days
  - 30 mg/kg dose level also tested – data being analysed
- Anxiety, locomotor and social activity, learning and species typical behaviors were tested
  - Open field with habituation tests at 10 minutes and 24 hours
  - Successive alleys test
  - Elevated plus maze
  - Contextual fear conditioning
  - Social behavior
- Anatomical aspects of the phenotype were also measured
  - Dendritic spine density in the hippocampus (in vitro; 0.5, 5, 50 nM)
  - Testis weight

NNZ-2566: Fragile X initial results

- Anxiety and locomotor activity
  - *fmr1* KO mice show hyperactivity in the open field, Successive Alleys and Elevated Plus Maze tests
  - NNZ-2566 reduces this hyperactivity

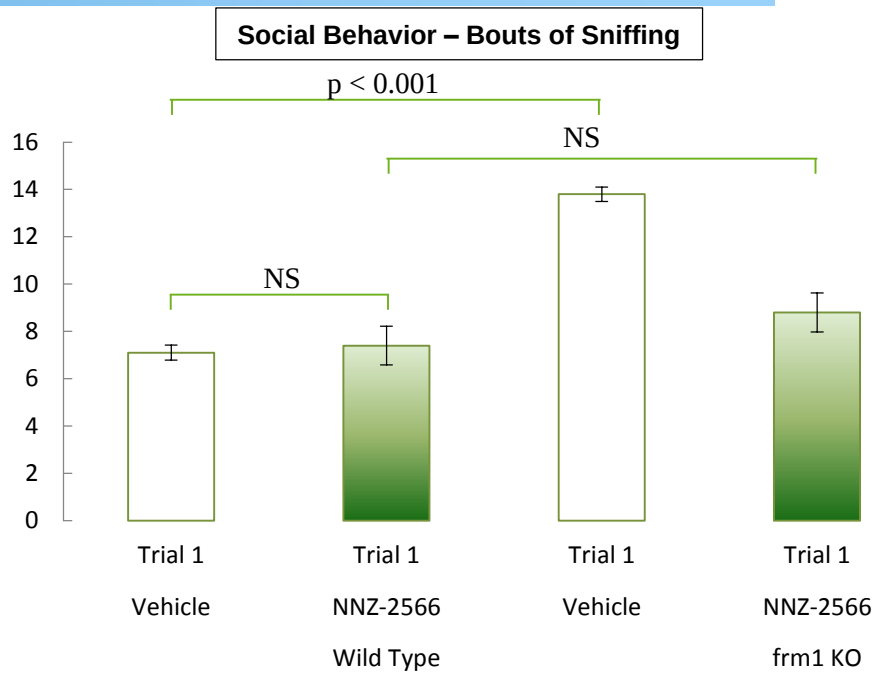


- Learning
  - *fmr1* KO mice show a deficit in habituation (short term memory) which is reversed by NNZ-2566
  - *fmr1* KO mice show a deficit in contextual fear conditioning which is reversed by NNZ-2566

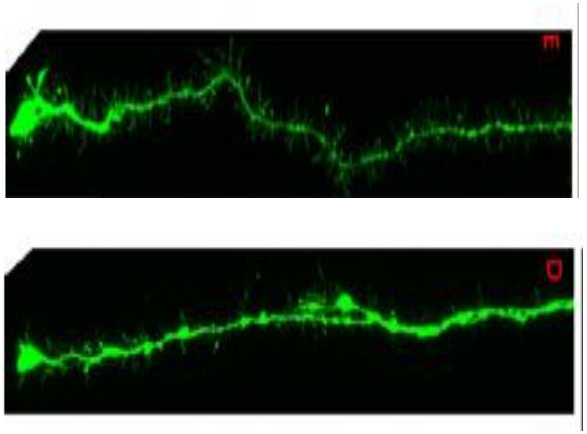


NNZ-2566: Fragile X initial results

- Social behaviors and complex species typical behaviors
  - *fmr1* KO mice show abnormal social interactions, which are reversed by NNZ-2566
  - *fmr1* KO mice show abnormalities in marble burying and nest building which are reversed by NNZ-2566



- CNS and peripheral morphology
  - *fmr1* KO mice show increased numbers of dendritic spines on hippocampal neurons which are reversed by NNZ-2566 in a dose-dependent manner in a concentration range of 0.5 – 50nM
  - *fmr1* KO control mice show macro-orchidism which is reversed by NNZ-2566



fmr1 KO + Vehicle

fmr1 KO + 50 nM NNZ-2566

Dendritic spine morphology (in vitro)

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