



INVESTOR PRESENTATION

9 October 2013

Dr Richard Treagus – Executive Chairman

Jon Pilcher – Chief Financial Officer

Forward Looking Statements

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 Snapshot

▣ ORIGINS

- Formed in 2001
- IP rights acquired from the University of Auckland
- Listed on the ASX in February 2005
- Partnership established with the US Army in 2009

▣ KEY STRATEGIC RELATIONSHIPS

- US Army Medical Research & Materiel Command
- International Rett Syndrome Foundation
- Fragile X Research Alliance
- Fragile X Drug Validation Initiative

▣ OPERATIONS

- New Zealand domiciled
- Finance and shareholder relations relocated to Melbourne
- Clinical development consolidated in the US



Leadership

BOARD OF DIRECTORS

- ▣ Richard Treagus (Executive Chairman)
 - Former CEO Acrux Limited
- ▣ Trevor Scott
 - Head of Audit Committee
- ▣ Bruce Hancox
 - Former Group CEO Brierley Investments Limited
- ▣ Larry Glass

MANAGEMENT

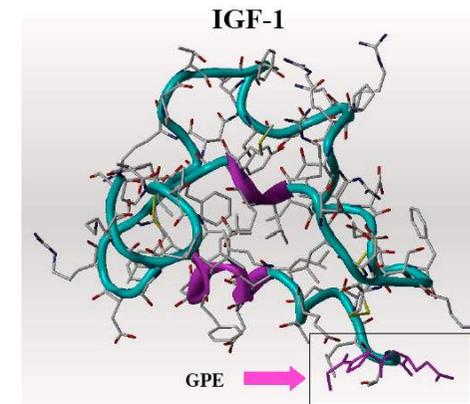
- ▣ Larry Glass (CSO)
 - 9 years with Neuren
- ▣ Jon Pilcher (CFO)
 - Former CFO Acrux Limited
- ▣ James Shaw (COO)
 - Former Quintiles Asia-Pac
- ▣ Joe Horrigan (CDMA)
 - Neuropsychiatrist
 - Former Head of Medical Research at Autism Speaks

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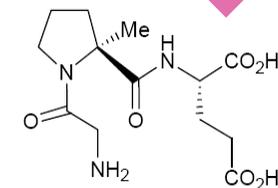
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Scientific Foundation

- ❑ **IGF-1** is a naturally occurring growth factor in the brain
 - Produced following brain injury and stress
 - One of the brain's self-repair mechanisms
- ❑ **Glypromate** (GPE) is considered the active part of the molecule
- ❑ **NNZ-2566** is a synthetic analogue of Glypromate
- ❑ **NNZ-2566** influences the processes underlying acute and chronic CNS disorders
 - Inflammation
 - Microglial function
 - Synaptic plasticity (inter-neuronal communication)
 - Abnormal electrical activity (e.g., seizures)
- ❑ **NNZ-2566** potentially treats a wide range of neurological conditions
- ❑ **NNZ-2591** is in the same class of peptides

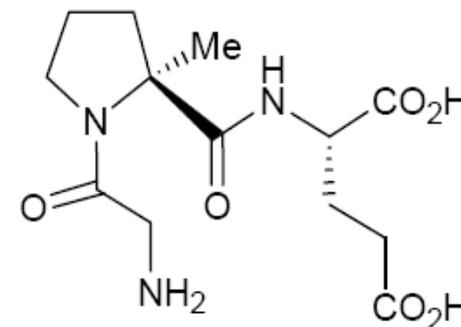


NNZ-2566



NNZ-2566

- Pre-clinical efficacy has been demonstrated in animal models of TBI, stroke, cognition, Fragile X and Rett Syndromes
- Administered **intravenously** or **orally**
- 40-50% orally bioavailable, with or without food
- Crosses the blood brain barrier
- Well tolerated at high doses
- Validated manufacturing process in the USA
- Patent protected

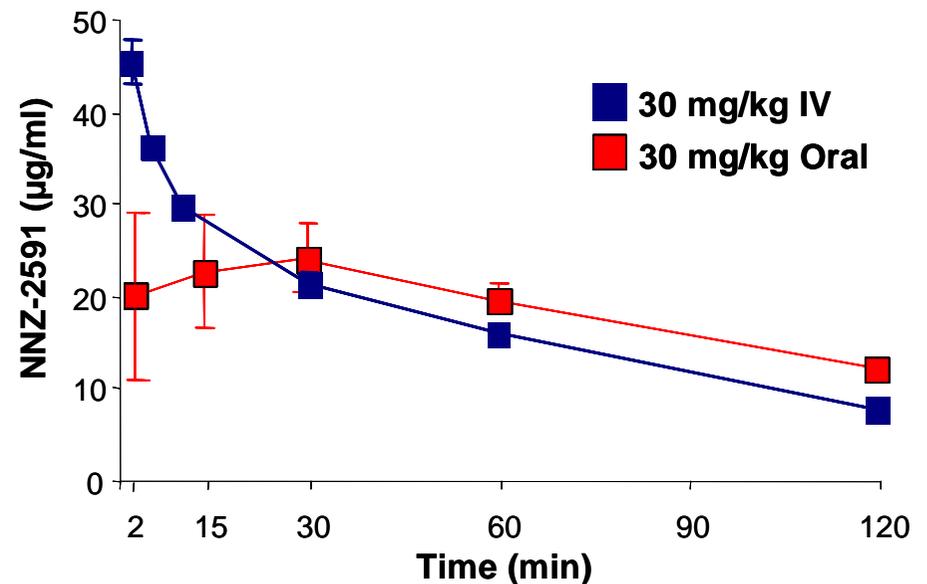


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NNZ-2591

- Synthetic analog - cyclic dipeptide
- **100%** orally bioavailable
- Testing in Fragile X model completed; **results comparable to NNZ-2566 at 1/3rd the dose**
- US Army working on mechanism of action under Cooperative R&D Agreement



Intellectual Property

- ▣ Broad patent estate with no royalties payable

- ▣ **NNZ-2566** and other GPE analogues
 - 7 issued patents covering composition, oral formulation and methods of use
 - 7 pending applications
 - Remaining patent life between 9 and 15 years
 - Additional market exclusivity may be available via Orphan and Pediatric Drug designations

- ▣ **NNZ-2591** and other bicyclic analogues
 - 3 issued patents covering composition, formulation and methods of use
 - 3 pending applications
 - Remaining patent life between 11 and 15 years

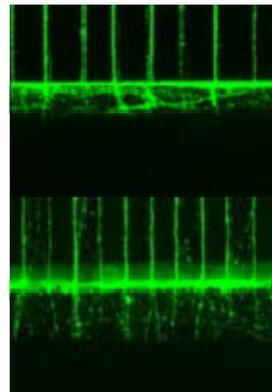
Pre-Clinical Studies



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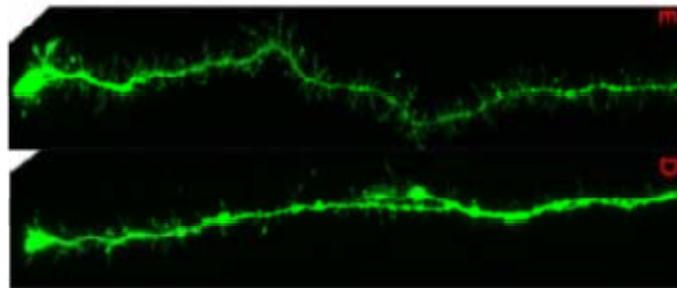
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Dendritic Spines - NNZ-2566 in Fragile X (*fmr1*) model



Wild-type

***fmr1* knockout**



***fmr1* knockout + Vehicle**

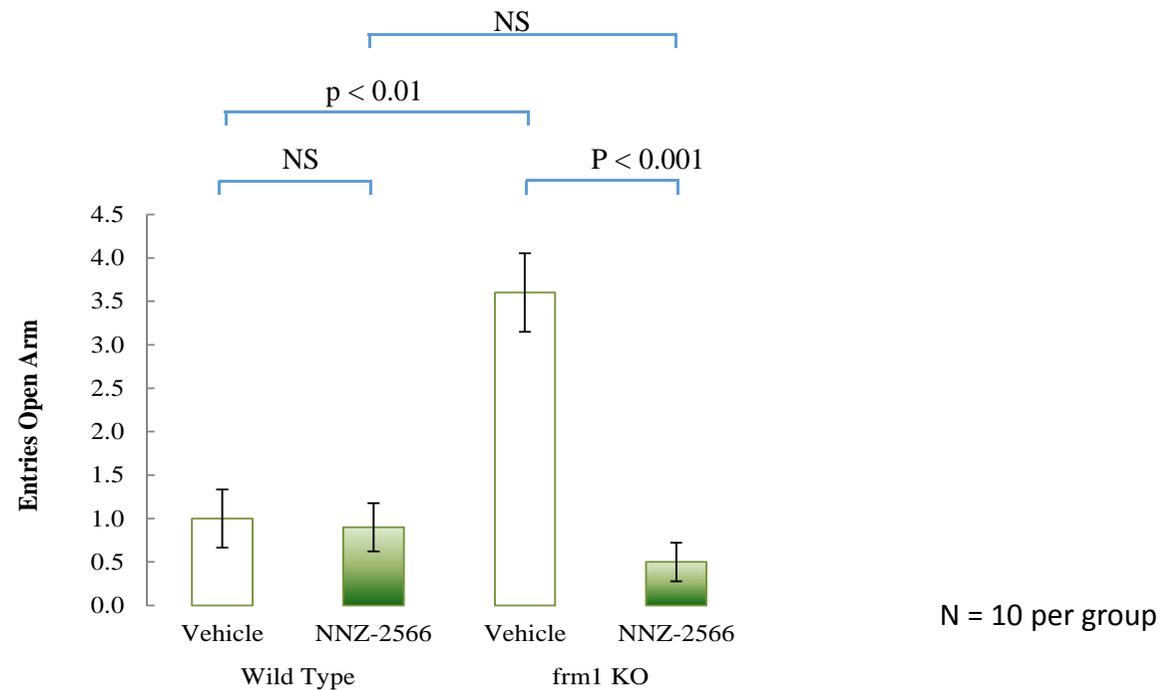
***fmr1* knockout + NNZ-2566 (50 nM)**

Dendritic spine morphology

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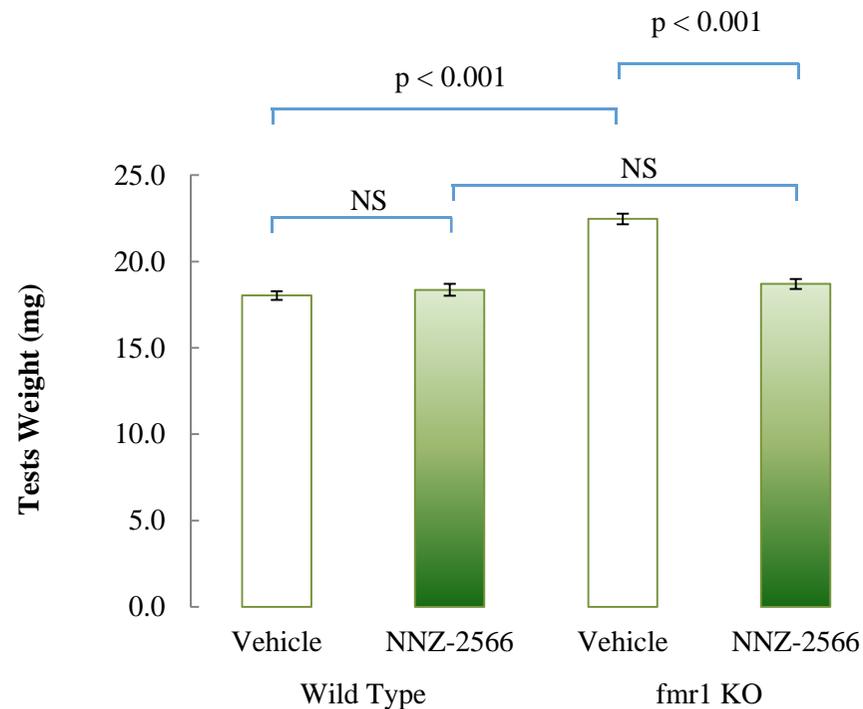
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Behaviour - NNZ-2566 in Fragile X (*fmr1*) model



- *fmr1* KO mice show a significant difference to Wild Type controls
- NNZ-2566 treated *fmr1* KO mice are not significantly different from vehicle treated Wild Type mice
- NNZ-2566 significantly normalizes behaviour of *fmr1* KO mice

Enlarged testes - NNZ-2566 in Fragile X (*fmr1*) model



- *fmr1* KO mice show an increase in testis weight, as do human Fragile X Syndrome patients
- After NNZ-2566 administration to *fmr1* KO mice, testis weight is not significantly different from controls
- NNZ-2566 has no significant effect in Wild Type mice



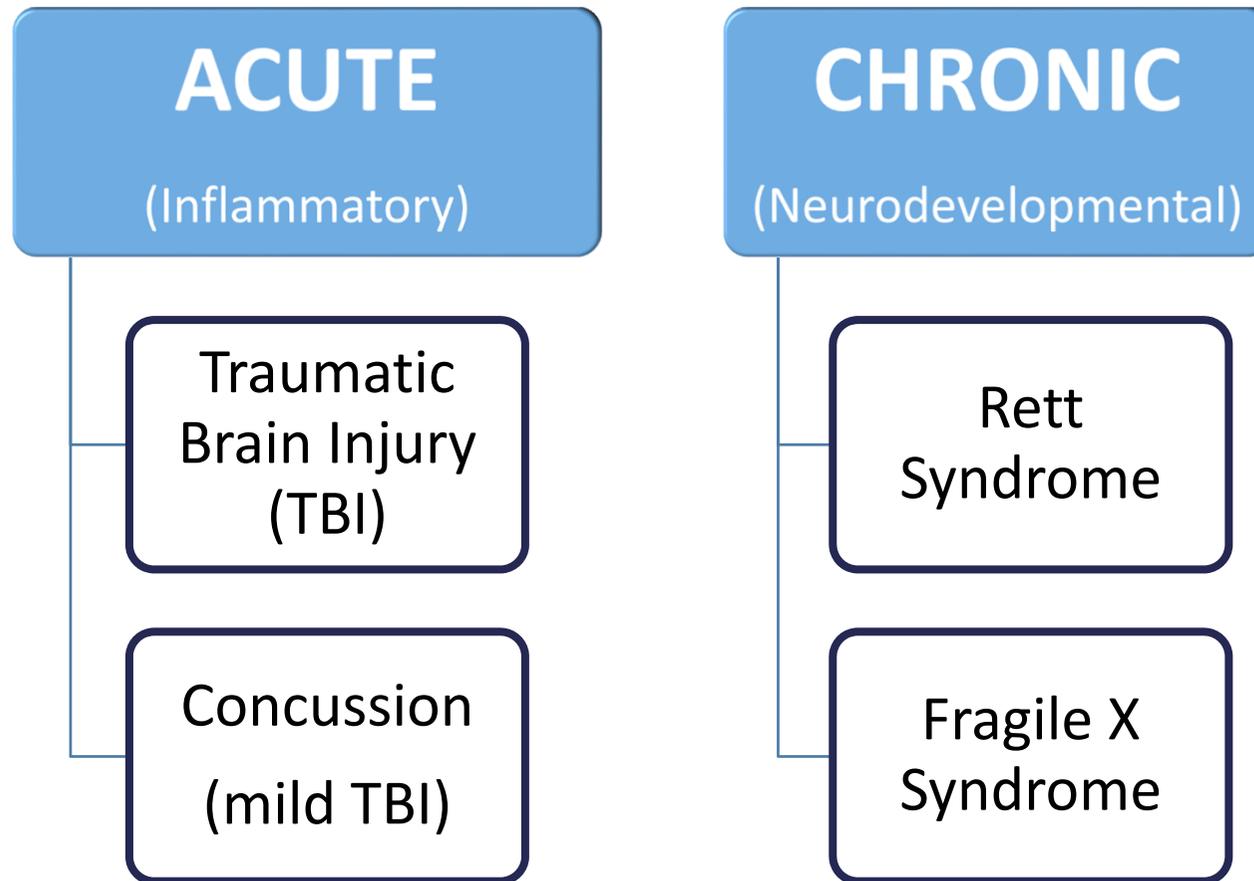
Key Clinical Programmes



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NNZ -2566 Clinical Strategy



TBI and Concussion

- NNZ-2566 improves functional recovery, preserves cognitive function, inhibits post-injury seizures
- > 1.5 million head injuries annually in the US alone; >75% are mild or concussion
- Leading cause of death and disability, especially in young people and the elderly
- Partnership funding of ~US\$23 million by US Army
- Only late-stage competition is progesterone
- \$4+ billion estimated global market potential

Traumatic Brain Injury (*INTREPID*)

- Phase II randomized (2:1), placebo-controlled, fixed dose escalation, safety and efficacy study
- 260 subjects, ages 16-75 with moderate to severe TBI (Glasgow Coma Scale 4-12)
- NNZ-2566 IV solution or placebo administered within 8 hours of injury as a 20 mg/kg bolus followed by 72 hours of infusion at 1, 3 or 6 mg/kg/hr (30, 30 and 200 subjects, respectively)
- Endpoints: safety, functional status at 1 and 3 months, cognitive and neuropsychological function at 1 and 3 months, non-convulsive seizures and biomarkers in the first 5 days
- 2 protocols: LAR and EFIC
- Goal: 18 Level I and II US trauma centers with 10 under EFIC
- 122 subjects enrolled to date

Concussion (mTBI)

- Phase II randomized (1:1:1), placebo-controlled, fixed dose safety and efficacy study
- 132 subjects, 18-55 with mild TBI (Glasgow Coma Scale 13-15) and a pre-injury computerized neurocognitive assessment
- NNZ-2566 oral solution or placebo administered within 24 hours of injury at 35 or 70 mg/kg twice daily for 7 days
- Endpoints: safety, change from baseline cognitive function and time to return to pre-injury baseline cognitive function assessed at 1, 2, 4 and 8 weeks
- Sites: 1-2 US military training facilities
- Start date to be determined

Rett Syndrome

- Mutation in a gene on the X chromosome
- 1 / 10,000 females (20,000 USA)
- Most physically disabling of the autism spectrum disorders
- Many girls live into adulthood, requiring total 24-hr-day care
- Profound disability and financial burden for >50,000 patients and families globally
- Symptoms include:
 - Loss of speech and motor control
 - Compulsive hand movements
 - Disorders of breathing and cardiovascular function
 - Extreme anxiety
 - Seizures

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Rett Syndrome Phase II (RTT-001)

- Phase II randomized (2:1), placebo-controlled, dose escalation, safety and efficacy study
- Up to 60 (48 complete) female subjects ages 16-40 years
- Subjects stratified 1:1 based on mutation (correlates with severity)
- NNZ-2566 oral solution or placebo administered for 28 days following 3 days of dose titration up to 35 or 70 mg/kg b.i.d.
- Endpoints: safety, seizure activity, cardiac and respiratory irregularities, caregiver and clinician assessments of symptom severity and behaviour
- Sites: Rett Syndrome Centers at Baylor College of Medicine and University of Alabama
- 17 subjects enrolled to date; 10 have completed the entire study

Fragile X Syndrome

- Mutation on the X chromosome affecting both males and females
- The most common inherited cause of intellectual disabilities and the most common known cause of autism
- 1 / 4,000 males and 1 / 6,000 females (58,000 USA)
- Symptoms include:
 - Intellectual disabilities
 - Attention deficit, hyperactivity and autistic behaviour
 - Anxiety and unstable mood
 - Seizures (approximately 1 in 4)

Fragile X Syndrome Phase II (FXS-001)

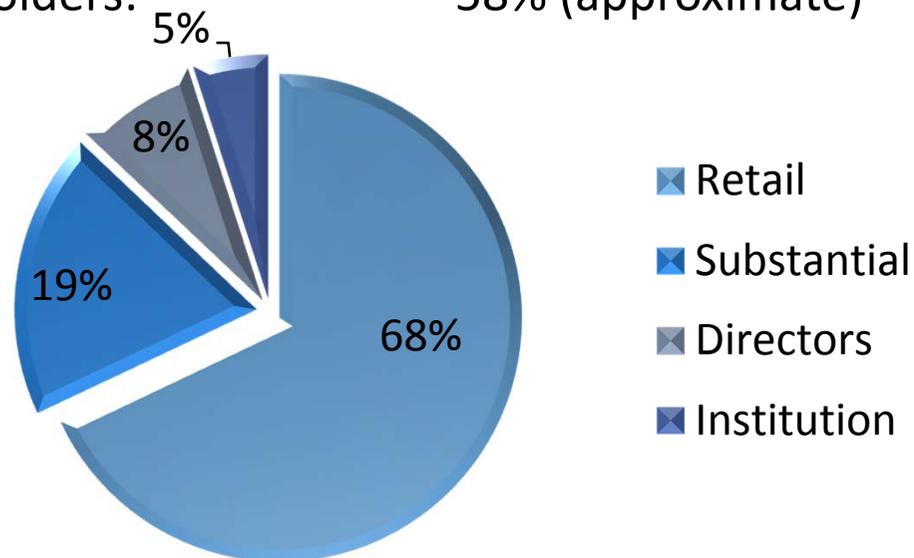
- Phase II randomized (1:1:1), placebo-controlled, fixed dose, safety and efficacy study
- Up to 72 (60 complete) male subjects ages 16-40 years
- NNZ-2566 oral solution or placebo administered for 28 days at 35 or 70 mg/kg b.i.d
- Endpoints: safety, seizure activity, caregiver and clinician assessments of symptom severity and behavior, biomarkers
- Sites: Rush University Medical Center and up to 4 other Fragile X Syndrome centers
- To be initiated before the end of 2013

Strategy

- ▣ Demonstrate the therapeutic benefit of **NNZ-2566** in human subjects
- ▣ Expand treatments to include both acute and chronic conditions
- ▣ Potential to establish a “**gateway**” to autism and other neurodevelopmental disorders
- ▣ Criteria for selecting therapeutic targets
 - Significant unmet need and commercial opportunity with no approved drugs
 - Regulatory advantages – eligible for *Fast Track, Orphan Disease, Breakthrough Therapy*
 - Strong support from advocacy groups and other stakeholders
- ▣ Realising value
 - Elicit a “clinical signal” with NNZ-2566 in human subjects
 - Advance the pre-clinical work with NNZ-2591
 - Maintain dialogue with potential partners

Shareholdings and Financial Position

Shares outstanding:	1.25 billion
Options outstanding:	255 million
Closing price 4 Oct 2013	10.5 cents
52 week range:	2.2 cents - 12.5 cents
Market capitalization 4 Oct 2013	\$131 million
Cash at 30 June 2013	NZ\$4.1 million
Top 20 shareholders:	58% (approximate)



Investment Summary

- ▣ **Patented drug analogues** of naturally occurring brain growth factors
- ▣ Potentially applicable to both **acute** and **chronic** neurological conditions – with large unserved markets.
- ▣ **Compelling pre-clinical efficacy data** in TBI, Fragile X and Rett Syndrome models
- ▣ Abbreviated regulatory pathways - with possible **Orphan drug** designation
- ▣ Experienced clinical and commercial management team
- ▣ Phase II clinical trials will report results from mid-2014
- ▣ Clinical data will provide the basis for partnering discussions

Expected News Flow

Initiate Fragile X Phase 2	2H-2013
Complete enrollment in Rett Phase 2	1H-2014
Top-line results for Rett Phase 2	2H-2014
Complete enrollment in Fragile X Phase 2	2H-2014
Complete enrollment in <i>INTREPID</i>	2H-2014
Top-line results for Fragile X Phase 2	1H-2015
Top-line results for <i>INTREPID</i>	1H-2015