Improving Lives

Neurotech International

Rett Syndrome Clinical Trial Results

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6 May 2024

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Neurotech Four Core Strategies

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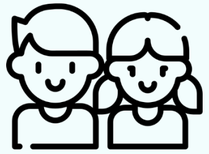
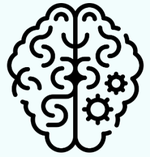
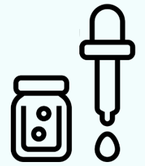


Focus on Paediatric Patients



Focus On Rare Neurological Disorders with Neuroinflammation

Focus on Partnering with Key Opinion Leaders / Clinicians



Focus On Drug Product Development

Clinical Pipeline – 2024

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Pre-Clinical

NTI164
Combination Therapies
Prednisone, Diclofenac, Other

Other Licensed Strains

Phase I/II

NTI164
Cerebral Palsy

NTI164
PANDAS / PANS[1]

NTI164
ASD
(90 week+ open label extension)

NTI164
Rett Syndrome

Phase II/III

NTI164
ASD

Pipeline (2020/1)

NTI164
Combination Therapies
Prednisone, Diclofenac, Other

NTI164
Neuronal Cell Assays

Other Licensed Strains

Data reported (all with statistically significant primary endpoint results)

1. Paediatric Autoimmune Neuropsychiatric Disorders Associated with Streptococcal Infections (PANDAS) and Paediatric Acute-Onset Neuropsychiatric Syndrome (PANS)

Rett Syndrome Phase I/II Trial

“Caregivers of children with RTT experience the illness as being like an “obstacle course”, where they must continuously overcome hurdles. These include hindrances for finding responses to their symptoms and achieving a diagnosis, for managing the treatment and daily care, and for finding the essential financial resources to meet all the expenses generated by the illness.”[1]

1. Palacios-Ceña D, Famoso-Pérez P, Salom-Moreno J, Carrasco-Garrido P, Pérez-Corrales J, Paras-Bravo P, Güeita-Rodriguez J. “Living an Obstacle Course”: A Qualitative Study Examining the Experiences of Caregivers of Children with Rett Syndrome. International Journal of Environmental Research and Public Health. 2019; 16(1):41

Rett Syndrome Trial Design (NTIRTT1)

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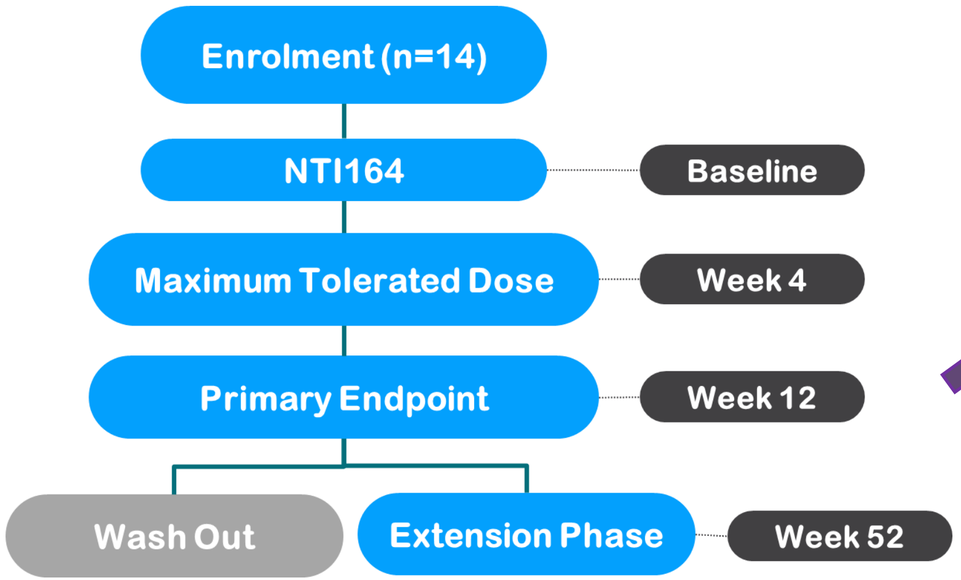

All patients completed 12 weeks i.e. no adverse events requiring withdrawal from the study

All patients entered the 52 week extension phase of the study

High potency, Broad Spectrum Cannabinoid Formulation in Oil, *C. sativa L.* (Plant Derived)

- Entourage Effect
- Neuroprotective
- Anti- Neuroinflammatory

Primary Endpoint

- Clinical Global Impression – Improvement (CGI-I)

Secondary Endpoints

- Rett Syndrome Behaviour Questionnaire (RSBQ)
- CGI-severity of illness (CGI-S)
- RTT- Clinician Domain Specific Concerns – Visual Analog Scale (RTT-DSC-VAS)
- Impact of Childhood Neurological Disability Scale (ICNDS)
- Overall Quality of Life Rating of the Impact of Childhood Neurological Disability Scale (ICNDS-QoL)
- Rett Syndrome: Symptom Index Score (RTT-SIS)
- RTT Caregiver Burden Inventory (RTT-CBI)
- Safety
- Communication and Symbolic Behaviour Scales Developmental Profile™ Infant-Toddler Checklist (CSBS-DP-IT Social)

* No participants received DAYBUE™ (trofinetide)[1]

1. DAYBUE is a trademark of Acadia Pharmaceuticals Inc

Baseline Patient Characteristics

Characteristic		Number (%) / Mean
Age		8.8 years
Weight		27.5 kg
Sex	Female	14 (100%)
CGI-S	Mean	4.6 (100%)
	Moderate (4)	7 (50%)
	Marked (5)	5 (36%)
	Severe (6)	2 (14%)
RSBQ Total Score	Mean	44.6 (100%)
	<35	2 (14%)
	≥35	12 (86%)

A total of 14 female patients with Rett Syndrome participated

12 Week Safety Data

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NTI164 Exhibits Excellent Safety Over 12 Weeks

A total of 14 patients evaluable at 12 weeks

One serious adverse event (SAE) recorded (Urticaria-hives) Across all doses, across entire period (12 weeks)

Adverse events (AEs) were tolerated and manageable 11 AEs*, 4 patients

Weight Loss/Gain

- No change from baseline

Vomiting^

- 2 pts (14%)

Diarrhoea

- 0 pts (0%)

	Weight Loss/Gain	Vomiting^	Diarrhoea
DAYBUE™	12% with >7% weight lost	29%	82%

Kidney/Liver Function

Vital Signs

Blood Chemistry

Normal

Conclusion: NTI164 exhibits an excellent safety profile and minimal patient-specific side-effects
(consistent with autism and PANDAS/PANS clinical data)

*Other AEs were common cold, viral infection, pharyngitis , chest infection.
^None of these adverse events were serious and were not considered to interfere with the patient's functioning. No additional treatment was required (i.e. administration of anti-vomiting medications). DAYBUE data, source: Acadia Pharma.

Summary of Efficacy Measures

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Primary Endpoint

- **CGI-I**

Improvement of 10% from Baseline at 12 weeks (p=0.009) – 9 exploratory Rett-Specific Anchors
Improvement of 23% from Baseline at 12 weeks (p=0.001) – 4 core Rett Anchors (further development)

FDA APPROVED

- CGI-I
- RSBQ

→ Co-primary endpoints used for FDA approval in trofinetide Phase 3 trial

Secondary Endpoints

- **RSBQ** — Patients receiving NTI164 showed a **13.4** score decrease in average RSBQ total score versus baseline (30% improvement, p<0.001)
- **CGI-S** — Patients receiving NTI164 showed a **0.4** decrease in CGI-S versus baseline (8.7% improvement, p=0.009)
- **ICNDS** — Patients receiving NTI164 showed an **8.5** score decrease versus baseline (13% improvement, p=0.004)
- **ICNDS-QoL** — Patients receiving NTI164 showed a **1.5** score increase versus baseline (60% improvement, p<0.001)
- **RTT-CBI** — Patients receiving NTI164 showed a **5.0** score decrease versus baseline (16% improvement, p=0.025)
- **RTT-DSC-VAS** — Patients receiving NTI164 showed a 0.6 score decrease in verbal communication (13% improvement, p=0.014) but no improvement in ambulation (p=0.374), communication choices (p=0.374) and hand function showed no change (NM)
- **RTT-SIS** — No significant change (p=0.146) in total score

CSBS-DP-IT — **Not Measured**

Rett Syndrome Behavioural Questionnaire RSBQ is a caregiver-completed scale assessing a wide range of neurological and behavioural symptoms in RTT (maximum score 90). RTT Symptom Index Score RTT-SIS developed by A/Prof Ellaway examines 16 Rett-specific symptoms. RTT-Clinician Domain Specific Concerns-Visual Analog Scale RTT-DSC-VAS is a clinician-completed VAS assessing the severity of concerns in: (1) hand use; (2) ambulation; (3) seizures; (4) autonomic features; (5) behaviour; (6) attentiveness; (7) social interaction; and (8) language/communication. CGI-S is a single-item, 7-point scale by clinicians designed to assess global impression of severity (1=normal, 7 = severely ill). Impact of Childhood Neurologic Disability Scale ICNDS measures the impact that a child's condition has on the child's and the family's everyday life at the time of assessment and during the previous 3 months. It is an accurate, quick measurement tool reflecting the impact of behaviour, cognitive learning ability, physical/neurologic disability, and epilepsy on children and their families (0 = no impact, 132 = severe impact). ICNDS-QoL measures the overall quality of life of the affected individual (1 – poor, 6 –excellent). RTT-Caregiver Burden Inventory RTT-CBI is a syndrome-specific, caregiver-completed questionnaire that is based on the CBI designed for Alzheimer's disease (0= never, 5 = nearly always). N/M – not measured as no difference

Clinician Expert View on Results

Associate Professor Carolyn Ellaway – Lead Investigator

"The NTIRTT1 clinical trial is the first time a broad-spectrum cannabinoid drug therapy (NTI164) has demonstrated significant patient improvements in Rett Syndrome using validated clinical measures including CGI-I and RSBQ. Our data is very encouraging as we have observed clinically meaningful improvements in those symptoms repeatedly deemed as most important for treating clinicians, caregivers and patients; notably communication, hand behaviours, anxiety/mood and quality of life. These benefits have not compromised patient safety, with NTI164 displaying an excellent safety profile over the 12 weeks of the trial."

Established the Rett Syndrome Multi-Disciplinary Management Clinic, The Children's Hospital at Westmead.

Caregiver Testimonials

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Caregiver #1
“She seems much more in tune to what’s going on around her, e.g. patting the dog (has NEVER done this before)”

Caregiver #2
“She uses eye pointing, sometimes brings food / drink to an adult - this has not happened before”

Caregiver #3
“Since being on the trial, she will sit and listen to music for up to an hour”

Caregiver #3
“Since being on the trial, she is now trying to get our attention ”

Primary Endpoint: CGI-I

Clinical Global Impression – Improvement (CGI-I) is a 7–point scale that reflects experts' clinical judgment of the patient based on the clinician's total experience with the Rett syndrome population graded from 1 (very much improved) to 7 (very much worse). A decrease in CGI-I score indicates improvement.

CGI-I Primary Endpoint Significantly Improved

17 April 2024 - Reported

Top-Line CGI-I : 4 anchors of 9 available, assessed and reported

CGI-I versus baseline mean difference of - 0.3 (95% CI -0.015, -0.56; p = 0.04)

6 May 2024 - Reported

Complete CGI-I : 9 anchors of 9 available, assessed and reported

CGI-I versus baseline mean difference of - 0.4 (95% CI -0.112, -0.681; p = 0.009)

Rett-Specific Anchors	Top-line	Full
Communication		✓
Mental Alertness		✓
Hand Use		✓
Socialisation / Eye Contact		✓
Alertiveness	✓	✓
Anxiety	✓	✓
Autonomic	✓	✓
Seizure Activity	✓	✓
Sleep		✓

Clinical Global Impression (CGI) - is a physician/observer-rated scale synthesizing the clinician's impression of the global state of an individual & frequently employed in clinical trials for neuropsychiatric disorders. The CGI is a 3-item observer-rated scale that measures illness severity, global improvement and therapeutic effect.

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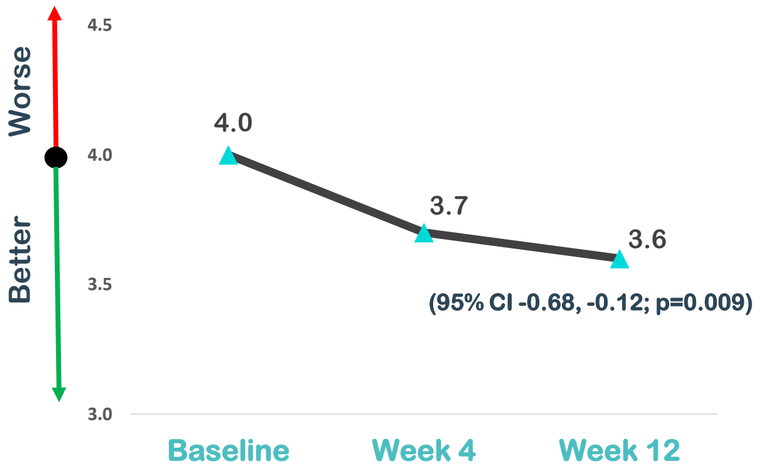
	Very Much Improved	Much Improved	Minimally Improved	No Change	Minimally Worse	Much Worse	Very Much Worse
Scale	1	2	3	4	5	6	7
NTI164 (week 4)	0 (0%)	0 (0%)	6 (43%)	7 (50%)	1 (7%)	0 (0%)	0 (0%)
NTI164 (week 12)	0 (0%)	0 (0%)	7 (50%)	6 (43%)	1 (7%)	0(0%)	0 (0%)

All 9 Rett-Specific Anchors



CGI-I improved 10% at 12 weeks (p = 0.009)

Clinical Interpretation

- Significant improvement seen by the treating clinician in 7 out of 14 patients with 50% minimally improved.

Clinical Global Impression (CGI) - is a physician/observer-rated scale synthesizing the clinician's impression of the global state of an individual & frequently employed in clinical trials for neuropsychiatric disorders. The CGI is a 3-item observer-rated scale that measures illness severity, global improvement and therapeutic effect.

CGI-I: Specific Rett Anchor Analysis

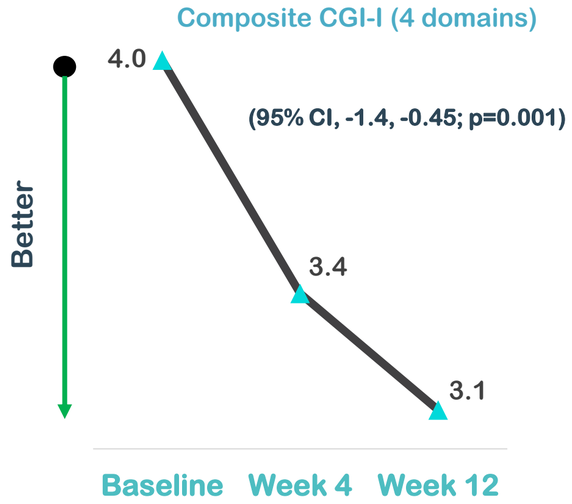
- As a first in human trial of NTI164 in Rett patients, Neurotech examined nine (9) anchors/sub-domains to further understand what domain benefits NTI164 could target for registration-directed trials
- Only 2-3 sub-domains are typically examined in Phase 3 trials for CGI-I: composite results for four (4) domains consistently cited by doctors, caregivers as important and where NTI164 showed strong improvements are shown

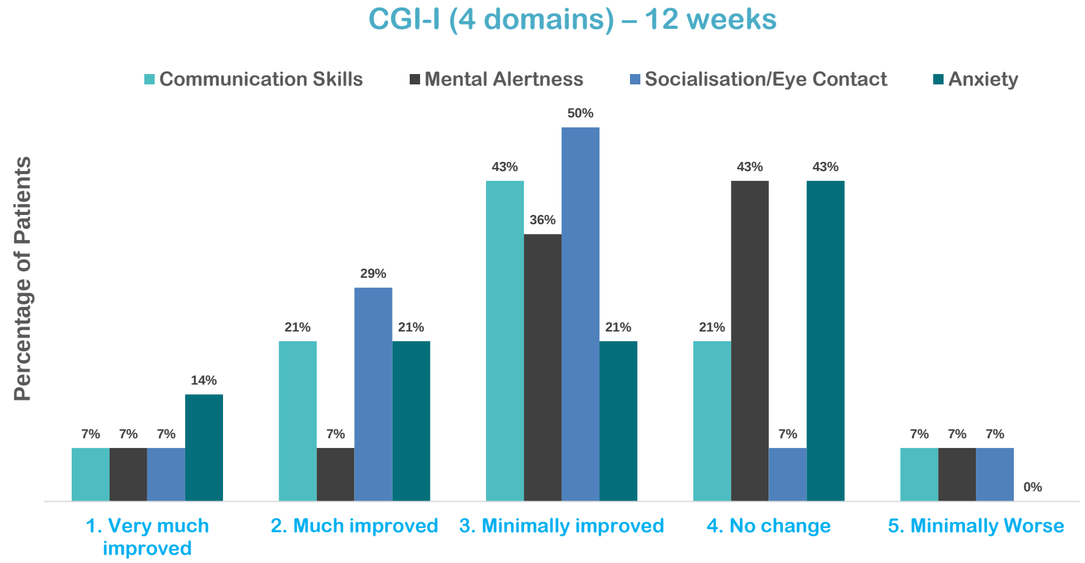
Communication Skills

Mental Alertness

Socialisation / Eye Contact

Anxiety





	Very Much Improved	Much Improved	Minimally Improved	No Change	Minimally Worse	Much Worse	Very Much Worse
Scale	1	2	3	4	5	6	7
NTI164 (week 12)	1 (7%)	4 (29%)	8 (57%)	1 (7%)	0 (0%)	0 (0%)	0 (0%)

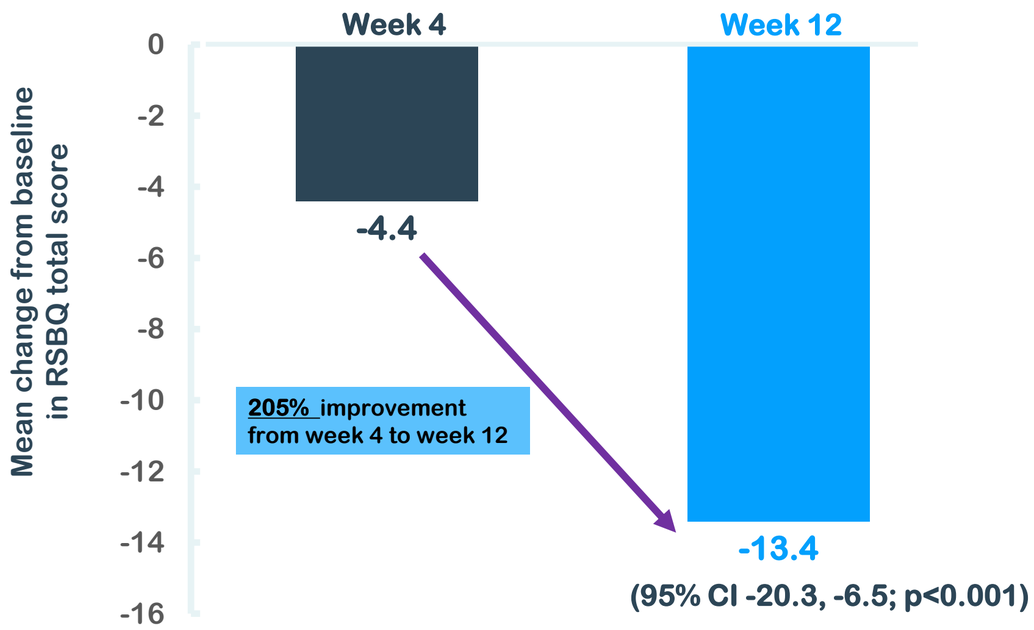
CGI-I (4 core domains) improved 23% at 12 weeks (p=0.001). 93% of patients improved

Clinical Global Impression (CGI) - is a physician/observer-rated scale synthesizing the clinician's impression of the global state of an individual & frequently employed in clinical trials for neuropsychiatric disorders. The CGI is a 3-item observer-rated scale that measures illness severity, global improvement and therapeutic effect.

Secondary Endpoint: RSBQ

The Rett Syndrome Behaviour Questionnaire (RSBQ) assesses the severity of neurobehavioral problems from the perspective of the caregiver and is one of the most widely used measures due to the specificity of its psychometric profile to the core features of Rett and is accepted by the United States Food and Drug Administration (FDA) for use in Rett Syndrome studies

Change from Baseline RSBQ Scores[1]



Total RSBQ Scores[1]

Baseline	4 weeks	12 weeks	P value
44.6	40.2	31.2	<0.001
Improvement (v baseline) mean diff.	4.4 (10%)	13.4 (30%)	

A lower score reflects lesser severity in signs and symptoms of Rett

RSBQ Sub Domain Scores[1]

Measure	12 weeks mean diff.	P value
Mood	-4.6	0.001
Breathing	-0.4	0.233
Hands	-2.0	<0.001
Face	-0.8	0.009
Body Rocking	-2.0	0.042
Nighttime	-1.0	0.161
Fear/Anxiety	-1.8	0.02
Walk/Stand	-0.8	0.104

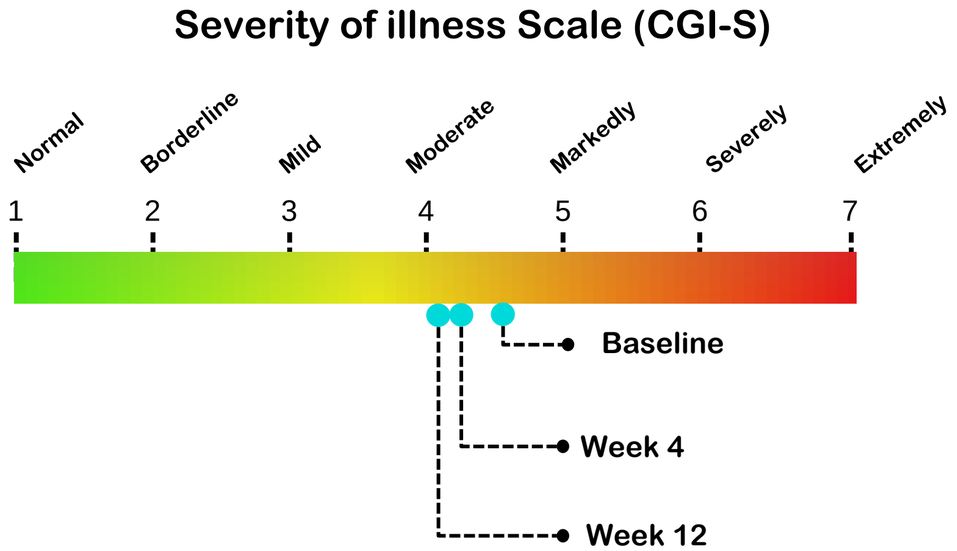
12 week RSBQ score improved 30% v baseline (p <0.001)

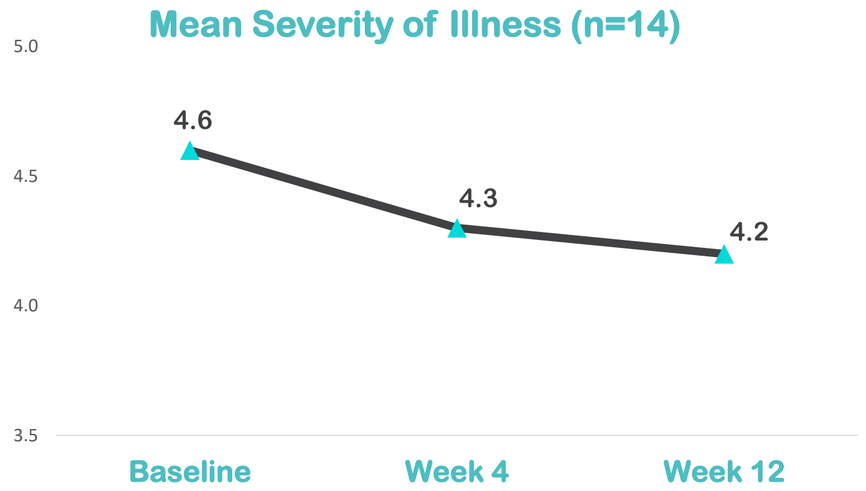
Clinical Interpretation

- Substantial 205% improvement from week 4 to week 12
- The change in RSBQ was aligned with CGI-I, implying that improvement in behavioural components may be related to overall clinical status

1. RSBQ was first shown to discriminate RTT from other intellectual disorders with good inter-rater and test–retest reliability scores. RSBQ consists of 45 items, rated as 0 = 'not true',1 = 'somewhat or sometimes true' or 2 = 'very true', that can be grouped into eight symptom domain subscales graded on a scale of 0–90 (maximum severity). 8 domains/subscales that reflect the core features of Rett examined: General Mood; Breathing Problems; Hand Behaviours; Repetitive Face Movements; Body Rocking and Expressionless Face; Nighttime Behaviours; Fear/Anxiety; and Walking/Standing. Moderate to high internal consistency has been reported for the total score and the 8 subscales, with good inter-rater and test–retest reliability scores, and significantly higher scores in a Rett population versus those with intellectual disability, thus validating its use as a diagnostic tool.

Secondary Endpoint: CGI-S





CGI-Severity of illness[1] (p = 0.009)

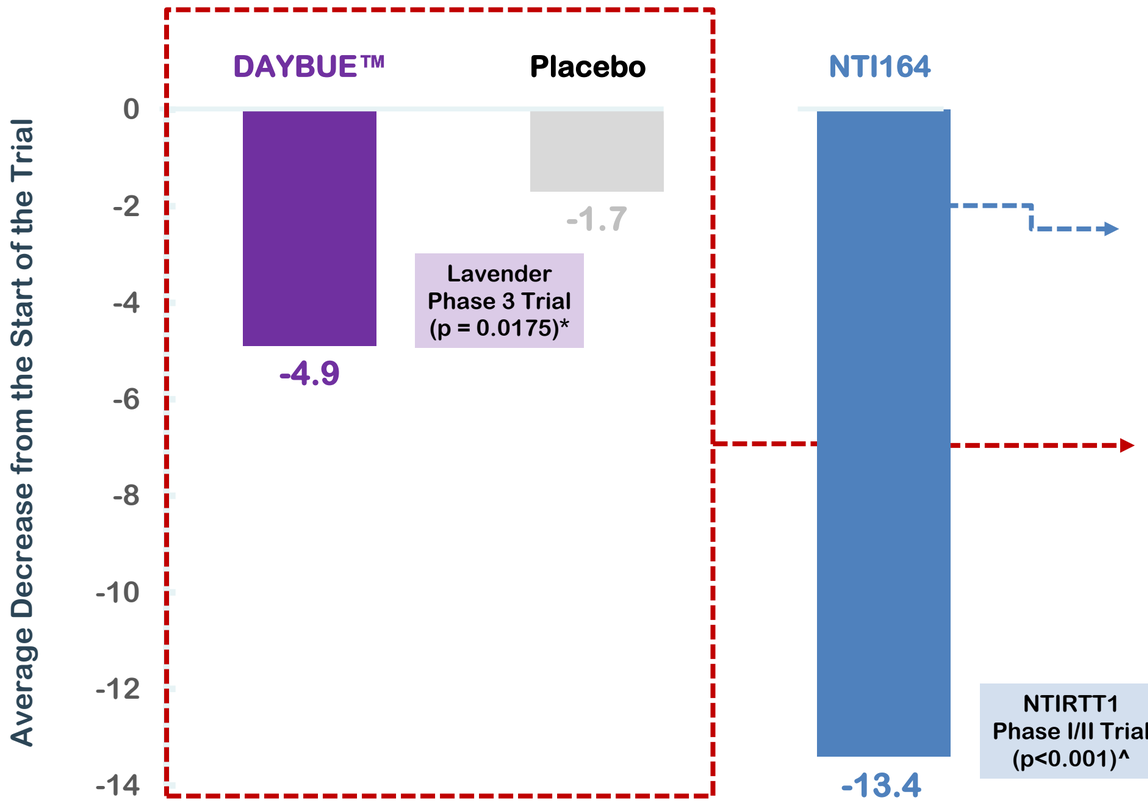
Clinical Interpretation

- CGI-S reduced to a more moderate state
- In other clinical trials to date, daily NTI164 continued to improve CGI-S beyond 12 weeks

1. Clinical Global Impression (CGI)- is a physician/observer-rated scale synthesizing the clinician's impression of the global state of an individual & frequently employed in clinical trials for neuropsychiatric disorders. The CGI is a 3-item observer-rated scale that measures illness severity, global improvement and therapeutic effect.

NTI164, DAYBUE™ RSBQ – 12 weeks

Rett Syndrome Behavioural Questionnaire (RSBQ)



- Patients receiving NTI164 showed a 13.4 score decrease in average RSBQ total score from the start of the trial when compared to baseline

*At commencement the average RSBQ total score for the NTI164 group was 44.6 (baseline) and 31.2 at 12 weeks **(30% RSBQ improvement)**.*

- Data from Lavender showed patients receiving DAYBUE saw a 4.9 score decrease in average RSBQ total score from the start of the trial versus baseline and 3.2 score decrease when compared with placebo

At commencement the average RSBQ total score for the DAYBUE group was 43.7 (11% RSBQ improvement) and 44.5 for placebo (4% RSBQ improvement)

For basic comparison purposes only – not directly studied in the same clinical trial.

Source: Company analysis, Acadia Pharmaceuticals

- *p value measured between the DAYBUE™ arm and the placebo arm using the least squares mean (LSM) change from baseline to week 12 versus placebo
- ^ p value measured between NTI164 at 12 weeks versus baseline (week 0) using paired sample T-test methodology for the observed mean

Other Secondary Endpoints

NTI164

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Statistical Significance Met

- ICNDS — p=0.004
- ICNDS (QoL) — p<0.001
- RTT-CBI — p=0.025
- RTT-DSC-VAS Verbal Communication — p=0.014

Not Statistically Significant

- RTT-SIS — p=0.148
- RTT-DSC-VAS Ambulation — p=0.374
- RTT-DSC-VAS Communication Choices — p=0.374

Not Measured

- CSBS-DP-IT — N/M
- RTT-DSC-VAS Hand Function — N/M

Statistical significance = p<0.05; Not significant = p≥0.05, N/M – not measured
RTT-DSC-VAS - The four ratings are stand-alone measures and not intended to be combined for a total score

Conclusion

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Met Primary Endpoint

NTI64 has demonstrated a statistically significant and clinically meaningful improvement in CGI-I (mean change, -0.4; p=0.009). When examining core domains, NTI164 showed 23% improvement at 12 weeks (p=0.001) and 93% of patients improved.

Met Majority of Secondary Endpoints

NTI64 has demonstrated a statistically significant and clinically meaningful improvement. Key measure of RSBQ improved 205% between week 4 and week 12

NTI164 Very Safe

Single serious adverse event (urticaria). Small number of adverse events, relating to vomiting, no weight loss, no diarrhoea observed. None of the adverse events were considered to significantly interfere with the patient's functioning and none of the adverse events required any additional medications (i.e. anti-vomiting)

Huge Unmet Need

Single FDA approved therapy: DAYBUE™ (trofinetide); need for additional safe and effective therapies

Key Milestones – NTI164

1H CY2024

- HREC/TGA Approval Cerebral Palsy Phase I/II Clinical Trial
- 24-week PANDAS/PANS Phase I/II Clinical Trial Data
- Rett Syndrome Phase I/II (14 girls) 52-week Extension HREC Approval
- Results of ASD Phase II/III Clinical Trial
- Top-line Rett Syndrome Phase I/II Clinical Trial data
- Results of Rett Syndrome Phase I/II Clinical Trial – full data
- Meeting outcome – TGA[1] Regulatory Advice
- Publications for ASD Phase I/II + pre-clinical NTI164 results
- Metabologenomic data from Phase I/II PANDAS/PANS Clinical Trial

2H CY2024

- Orphan Drug Designation USA – Rett Syndrome
- Orphan Drug Designation USA – PANDAS/PANS
- Orphan Drug Designation Europe – Rett Syndrome
- Orphan Drug Designation Europe – PANDAS/PANS
- Presentation of Phase I/II Rett Syndrome data at international Rett meeting
- FDA IND / EMA[2] toxicology
- Commence Phase I/II Cerebral Palsy Clinical Trial

1. Therapeutic Goods Administration (TGA)
2. Food and Drug Administration (FDA), European Medicines Agency (EMA)

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Appendices

Neurotech is a clinical-stage biopharmaceutical development company focused predominately on paediatric neurological disorders

Neurotech International

NTI164 exclusive worldwide licence for neurological disorders

Patents Pending – Use, Composition

Novel oral biopharmaceutical cannabinoid platform (NTI164)

Focus on Paediatric Patients

Multiple Phase I/II and Phase II/III Clinical Trials

Supportive Efficacy & Safety Data in Children

About Rett Syndrome

About

- Rare genetic neurological and developmental disorder and is almost exclusively the result of a mutation(s) in the methyl CpG binding protein 2 (MECP2) gene located on the X chromosome: **impaired brain development and function**
- Currently there is no cure for people with Rett syndrome and classified as a “rare/orphan disease” (by definition, less than 200,000 affected individuals in the US) by the Office of Rare Diseases of the National Institutes of Health

Neuroinflammation

- Numerous scientific reports support neuroinflammatory effects in Rett Syndrome
- NTI164 shown to exhibit anti-neuroinflammation and neuroprotective effects *in vitro*


MeCP2 deficiency exacerbates the neuroinflammatory setting and autoreactive response during an autoimmune challenge

M. I. Zalosnik[1,2], M. C. Fabio[3], M. L. Bertoldi[1,2], C. N. Castañares[3] & A. L. Degano[1,2]


Chapter 14

Microglia Involvement in Rett Syndrome

Noël C. Derecki, James C. Cronk, Jonathan Kipnis

First Ever Approval

Daybue™ (trofinetide)

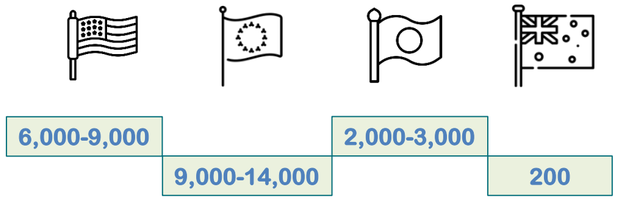
- Neuren Pharmaceuticals (ASX:NEU) / Acadia Pharmaceuticals (NASDAQ:ACAD): FDA Approval **10 March 2023**
- Sets benchmark for FDA accepted clinical endpoints, safety and tolerance

1. https://www.livewiremarkets.com/wires/a-de-risked-biotech-with-4x-upside
2. https://reverserett.org/about-rett-syndrome/

Rett Syndrome Market Dynamics

Neurotech International

Significant Market



- 17-26k patients in USA, Europe, Japan, Australia
- Est. US$2 billion annual market opportunity
- Narrow range of Rett specialist clinicians: focused prescriber group
- Concentrated market dynamics: 18 Rett Centres of Excellence in the US (3 in AU)
- No approved Rett drugs in Europe, Japan and Australia (USA:1)

Single Approved Therapy

Daybue™ (trofinetide)

- First FDA approved therapy (March 2023)
- Est. drug cost to patient ~US$1,000 per day. US$87 million in Q4 CY2023 (US$177m in CY2023) net sales
- Q3: 800 patient starts (4,500 registered with Rett, ~18% penetration) – strong demand highlights urgent market need
- CY2024 sales est. US$370m – US$420m

Valuation/Pricing Benchmarks

ACADIA™ neuren pharmaceuticals

- Neuren (ASX:NEU) license deal with Acadia (NASDAQ:ACAD) close to US$1 billion for trofinetide (*inc other indications)
- 80% covered lives for DAYBUE™ from US payers within 6 months – rapid reimbursement adoption
- Market approval via single Phase 3 clinical trial v placebo (“Lavender” – 187 pts), with open-label extension (“Lilac” – 154 pts)

Source: Acadia Pharmaceuticals, Neuren Pharmaceuticals Nov 2023 Investor Presentation, International Rett Syndrome Foundation, Rett Syndrome Association of Australia, Livewire Markets, Company Analysis

Therapeutic Agent: NTI164

High potency, Broad Spectrum Cannabinoid Formulation in Oil, *C. sativa L.* (Plant Derived)

- THC < 0.3%
- Major constituent Cannabidiolic acid (CBDA)
- Minor constituents include other cannabinoids: CBD, CBG, CBGA, other + terpenes

Convenient 1x or 2x (split dose) oral formulation in oil, ideal format for pediatric patients 20mg/kg (CBDA)

NTI164 is not a low dose CBD oil to be sold over-the-counter

Entourage Effect

Neuroprotective

Anti- Neuroinflammatory

Our Target Markets

Lack of effective therapies, significant unmet medical need

Annual Drug Therapy Market opportunity

Autism Spectrum Disorder (ASD)	Rett Syndrome	PANDAS/PANS	Cerebral Palsy (CP)
US$2 billion*	US$2 billion	US$1.4 billion[1]	US$4.3 billion
ndis	Orphan	Orphan	
$6.1 Billion			
Implied annual cost of ASD to NDIS based on average ASD funding of $32,800 per annum e.g. physio, psychology, speech therapy, support workers			
• Prevalence of ~2.0M <18 yr. patients in the US • 2 Approved Drugs (* limited use) • Risperidone, Aripiprazole	• Prevalence of ~15,000 patients in the US • 1 Approved Drug • Trofinetide	• Incidence of ~6,000 patients <18 yr. in the US[1] • No FDA/EMA Approved Drug	• Incidence of ~500,000 <18 yr. patients in the US • 2 Approved Drugs for spastic CP • Baclofen, Botox

1. Neurotech Estimate based on: Wald ER, et al. Estimate of the incidence of PANDAS and PANS in 3 primary care populations. Front Pediatr. 2023 Sep 21; EU/UK: 8,000 pts / US: 6,000 pts <18 years based on annual intravenous immunoglobulin (IVIG) cost of ~US$100k (https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8019941/)