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First Patient Dosed, Pivotal Study Begins

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Company Snapshot

Company	Neurotech International Limited (ASX: NTI)
Lead asset	NTI164, an oral cannabinoid medicine. It is a standardised extract from a proprietary cannabis strain, rich in CBDA (the natural acid form of CBD), with only 0.08% THC, so it does not cause a high.
Lead indication	Core symptoms of autism (ASD) in children with Level 2 or 3 autism, the moderate to severe end of the spectrum. Rett syndrome and PANDAS/PANS are follow-on indications.
Shares on issue	1,644.2m
Share Price (23 Sep 2026)	\$0.014
Market Capitalisation	\$23.0m
Net Cash (pro forma)	\$3.8m

Shares on issue include the 28.1m shares to be issued in relation to the 4 Aug placement. Net cash includes \$2.4m cash reported on 30 June minus the \$3.15m in R&D secured debt plus the \$4.5m raised in the 4 Aug placement.

In 2000, about 1 in 150 American children were identified with autism. The latest CDC figures put it at 1 in 31, and 1 in 20 boys. In Australia, autism is now the primary diagnosis for roughly 40% of the NDIS's ~740,000 participants. Yet no medicine in the US or Australia is approved to treat autism's core symptoms, meaning the social, communication and behavioural difficulties that define the condition. On 21 September Neurotech dosed the first child in a Phase 3 trial that aims to change that.

The two medicines approved for autism in the US, the antipsychotics risperidone (2006) and aripiprazole (2009), treat irritability and aggression rather than the condition itself, and both can cause weight gain and sedation.

Neurotech's approach rests on the idea that in many children with autism the brain's immune cells are over-active (neuroinflammation), and that calming this can improve day-to-day function. NTI164 is an oral, pharmaceutical-grade plant extract dominated by CBDA, with THC low enough to be non-intoxicating.

There is no approved core-symptom drug to benchmark against. The closest reference points are paediatric neurology drugs sold into much smaller populations. Jazz's Epidiolex, a CBD medicine for rare childhood epilepsies, sold US\$1.06b in 2025. Acadia's DAYBUE, the first approved drug for Rett syndrome, sold US\$391m in 2025 from a diagnosed US population of about 6,000 patients. Whether NTI can follow them to approval, in a far larger population, now rests on the Phase 3 trial that dosed its first child on 21 September.

Beyond Harmony Underway

Beyond Harmony is Neurotech's pivotal Phase 3 trial, which will support an application for approval. The first patient was dosed on 21 September 2026 and all six Australian sites are open (Victoria, Queensland, NSW,

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Tasmania and WA). The trial is randomised, double-blind and placebo-controlled, and aims to enrol up to 200 children with Level 2 or 3 autism under an adaptive design. That design lets the company make pre-set changes, such as adjusting the number of patients, after an interim look at the data. The trial registry lists a 16-week treatment period.

The main measure of success (the primary endpoint) is the change in the Vineland-3 Adaptive Behaviour Composite, a structured interview with parents or carers that scores everyday skills such as communicating, looking after oneself and getting along with others. Secondary measures include the SRS-2 (Social Responsiveness Scale, a questionnaire usually completed by a parent that rates social awareness, communication and repetitive behaviours) and two clinician ratings. The CGI-S rates how severe the child's condition is on a 1 to 7 scale, and the CGI-I rates how much the child has improved or worsened since starting treatment, also on a 1 to 7 scale.

Professor Michael Fahey, Head of Paediatric Neurology at Monash, is the Principal Investigator. The company reports more than 300 enquiries from families, so enrolment looks promising.

Beyond Harmony follows HARMONY, a 54-patient randomised, placebo-controlled Phase II/III trial run at a single site, Monash. HARMONY met its primary endpoint, with clinician-rated severity (CGI-S) improving significantly more on NTI164 than on placebo after 8 weeks with a p value less than 0.001 (meaning a difference this large would be very unlikely to occur by chance). Treated children moved on average from “markedly to severely ill” (5.54) to “mild to moderately ill” (3.77). The trial also showed statistically significant improvements over placebo on its secondary measures, including everyday functioning on the Vineland-3 ($p=0.024$), social responsiveness (SRS) and anxiety. The results were peer reviewed and published online in *Neurotherapeutics* in August 2026. The authors note the study's limitations, being a short 8-week blinded period, a small sample and a single site. Beyond Harmony is larger, multi-site, runs twice as long and uses Vineland-3 rather than CGI-S as its primary endpoint.

Two other workstreams run alongside the trial. First, the FDA cleared NTI164's IND in July 2026 so trials may be run in the US. The company plans a US population pharmacokinetics study under it, which measures how children absorb and clear the drug to help set dosing. Second, the Authorised Prescriber program, running since September 2025, lets specialists prescribe NTI164 outside trials at roughly cost price. It runs at seven sites with more than 200 patients referred and adds to the long-term safety record.

What a Positive Phase 3 Could Unlock

The aim is to show that NTI164 improves core autism symptoms by enough to support applications to the TGA and FDA. The company says the August raise funds it to topline data. The March quarterly noted discussions with pharma and biotech groups, some at detailed stages, with nothing binding. A positive result could also support the Rett and PANDAS/PANS programs, which rely on the same proposed mechanism, while a negative one would leave those earlier-stage programs carrying most of the company's value.

Recent Milestones

Date	Milestone
Sep 2025	Authorised Prescriber program launched in Australia
Oct 2025	FDA Rare Pediatric Disease Designation for NTI164 in Rett syndrome, adding to FDA (Nov 2024) and EU (Mar 2025) Orphan Drug Designations
Dec 2025 to Mar 2026	~A\$3.75m placement at 1.4c and A\$4.73m FY25 R&D tax refund received
Jan 2026	PANS clinical and mechanistic data published in Neurotherapeutics
Feb 2026	Ethics approval for Beyond Harmony, and a 90-day GLP dog study (a formal animal safety study) found no dose-limiting toxicity up to 216 mg/kg/day
Mar 2026	First Phase 3 site opened at Monash Children's Hospital
Jul 2026	FDA clears the US IND; USPTO Notices of Allowance on two US patents, expected to give protection to 2042 once granted; A\$3.15m R&D-refund-backed loan
Aug 2026	~A\$4.5m placement at 1.4c; Mark Davies appointed Executive Chairman; HARMONY results published online in Neurotherapeutics
8 Sep 2026	Andrew Chapman, founder of substantial holder Merchant Funds Management, joins the board as a Non-Executive Director
21 Sep 2026	First patient dosed in Beyond Harmony with all six sites open

Beyond Autism: The Broader NTI164 Pipeline

Rett syndrome is the most advanced follow-on program. An open-label Phase I/II study found NTI164 was well tolerated with no serious adverse events. The peer-reviewed paper (Journal of Paediatrics and Child Health, June 2025) covers 11 girls treated for 12 weeks. The company has separately reported 20-week extension data on 14 patients showing clinician-rated improvement of 33% versus baseline. NTI164 holds Orphan Drug Designation in the US and EU and Rare Pediatric Disease Designation in the US for Rett, which bring incentives such as fee waivers, tax credits and, on approval, seven years of US market exclusivity. Rett already has an approved drug in DAYBUE from Acadia, so NTI164 would need to show an advantage. The company points to better tolerability, but the two have not been compared directly.

PANDAS/PANS (childhood conditions where an immune reaction, often after a strep infection, triggers sudden OCD-like and other psychiatric symptoms) has completed an open-label Phase I/II study with statistically significant improvements. Results for a 14-child PANS sub-group were published in Neurotherapeutics in January 2026. Neurotech also holds ethics clearance for a Phase I/II study in spastic cerebral palsy.

Next Steps

Over the next year the focus is recruiting and dosing Beyond Harmony, deciding when to start the US dosing study and continuing partnering discussions.

Current Treatments and Commercial Benchmarks

Drug treatment for autism today targets related problems rather than the condition itself. In the US, risperidone and aripiprazole are approved for irritability in autism and both are available as generics. Johnson & Johnson, Otsuka and Bristol-Myers Squibb reported sales for the drugs overall but did not separately disclose revenue attributable to the autism indication. In Australia, Slenyto, a slow-release

melatonin tablet, has been approved since 2020 for insomnia in children with autism. Its developer, Neurim Pharmaceuticals, is private and does not publish sales. Doctors also prescribe medicines off-label (outside their approved uses), such as ADHD drugs for attention problems.

Paediatric neurology drugs with strong trial data and a defined population have reached far larger numbers. Epidiolex sold US\$1.06b in 2025, up 9%. DAYBUE (Acadia, licensed from ASX-listed Neuren) sold US\$391m in 2025, with 2026 guidance raised in August to US\$480-510m, and paid Neuren A\$65m in 2025 royalties. ADHD stimulants show the upper end. Vyvanse (Takeda) peaked at ~JPY459.3 billion (~A\$4.1b) in global sales in the year to March 2023 before losing patent protection, and J&J's Concerta reached US\$1.2b in 2008.

The Competitive Landscape in Autism

Neurotech is not alone in targeting autism, and the field has a long record of late-stage failures, often because children on placebo improve more than expected. In 2021 Servier stopped two Phase 3 trials of bumetanide in 422 children with moderate to severe autism after it showed no benefit on any measure, including Vineland and SRS. In September 2025 Harmony Biosciences' CBD gel ZYN002, a useful comparator to NTI164, missed its primary endpoint in a 215-patient Phase 3 in Fragile X syndrome (the most common single-gene cause of autism), which Harmony put down to a higher than expected placebo response. In June 2026 MapLight's ML-004 missed its primary social communication endpoint in a 161-patient Phase 2, though it showed a signal on irritability in adolescents.

Company	Drug	Target	Stage
Astrogen (private, South Korea)	AST-001, an L-serine syrup	Core symptoms in children	Phase 3 failed to establish efficacy; follow-on Phase 3 planned.
DeFloria (Charlotte's Web, AJNA, BAT)	AJA001, a full-spectrum CBD extract	Irritability in autism	Phase 2-ready; initiation subject to funding
Definium Therapeutics (Nasdaq: DFTX)	DT402, an MDMA-related compound	Core symptoms in adults	Phase 2a, first data expected 2026
SciSparc (Nasdaq: SPRC)	SCI-210, CBD plus a fatty-acid compound (PEA)	Autism symptoms in children	60-child randomised trial in Israel

Most of these programs target irritability or adults rather than core symptoms across the broad paediatric autism population, which is where Beyond Harmony sits. Astrogen is the closest direct competitor we found and used the same Vineland measure. Its Korean Phase 3 missed its 12-week primary endpoint, which the company put down to a larger placebo response than in Phase 2, and Korea's drug advisory committee voted against approval in July 2026. Astrogen is planning a further confirmatory trial and says it aims to file a US IND in October.

Deals, Policy and the Broader Market

Large drug companies have paid well for late-stage paediatric neurology and cannabinoid assets. Jazz paid US\$7.2b for GW Pharmaceuticals in 2021, with Epidiolex a key commercial asset in the acquisition. Epidiolex has since reached blockbuster status, demonstrating that a cannabinoid medicine can generate more than US\$1b in annual sales in severe childhood-onset epilepsies. Lundbeck bought Longboard Pharmaceuticals for US\$2.6b in October 2024, a 54% premium, largely for one Phase 3 drug for rare childhood epilepsies.

The Neuren and Acadia deal, built on royalties and milestones for DAYBUE, shows how an ASX developer can earn from a drug without building its own sales force.

On policy, autism has become a US political priority. In September 2025 US health officials promoted leucovorin (a folate drug) as a possible autism treatment and prescriptions surged. In March 2026 the FDA approved it only for a very rare folate transport disorder, saying there was not enough evidence for autism more broadly and calling for additional controlled studies. It showed strong public demand, and an FDA that still expects controlled trial data.

In Australia, the Thriving Kids reform (announced August 2025, with A\$2b of Commonwealth funding matched by the states) starts rolling out in October 2026, and from 1 January 2028 children under nine with mild to moderate autism are intended to access Thriving Kids rather than the NDIS. Beyond Harmony enrolls children with moderate to severe autism, so the direct link is limited, but the reform shows the pressure autism is placing on public funding.

Upcoming Catalysts

Catalyst	Expected timing	Significance
US dosing (PopPK) study starts under the cleared IND	Not guided	Starts the US part of the registration package
Beyond Harmony recruitment updates	Quarterly reports likely	Enrolment pace is the main driver of topline timing
Pre-specified adaptive interim analysis	Not disclosed	May change the sample size
Phase 3 topline data	Not guided	Primary efficacy readout for NTI164 in autism

Indicative timing only. Events may be delayed, modified or may not occur.

ASX Comparisons

ASX-listed companies in paediatric neurology, neurology and cannabinoid medicines.

Company (Ticker)	Lead Asset, Indication and Delivery	Clinical Stage	Market capitalisation (AUD, m)
Neuren Pharmaceuticals (NEU)	DAYBUE (trofinetide), Rett syndrome, oral solution (Acadia-partnered); NNZ-2591, Phelan-McDermid syndrome, oral	Marketed (DAYBUE); Phase 3 (NNZ-2591)	2,612.8
Alterity Therapeutics (ATH)	ATH434, Multiple System Atrophy (adults), oral	Phase 3-ready (FDA registration path agreed Jul 2026)	98.5
Avecho Biotechnology (AVE)	CBD soft-gel capsule, insomnia, oral (Sandoz-licensed for Australia)	Phase 3 (interim review Jun 2026 advised continuing unchanged)	77.3
Neurotech International (NTI)	NTI164, core autism symptoms, oral cannabinoid	Phase 3	23.0

Source: company disclosures. Iress 23 September 2026

Global Comparisons

Global listed companies in cannabinoid medicines, paediatric neurology and autism. Several of the autism programs sit inside larger companies whose value rests mostly on other drugs, so these are imperfect comparisons.

USD:AUD = 1.00:1.41

CAD:AUD = 1.00:1.00

Company (Ticker)	Lead Asset, Indication and Delivery	Clinical Stage	Market capitalisation (AUD, m)
Jazz Pharmaceuticals (NASDAQ: JAZZ)	Epidiolex, rare childhood epilepsies, oral CBD solution (plus sleep and cancer drugs)	Marketed (US\$1.06b 2025 sales)	22,019.3
Definium Therapeutics (NASDAQ: DFTX)	DT402, core autism symptoms in adults, oral (value driven by DT120 in anxiety)	Phase 2a	7,638.9
Acadia Pharmaceuticals (NASDAQ: ACAD)	DAYBUE, Rett syndrome, oral	Marketed (US\$391m 2025 sales)	6,416.3
Harmony Biosciences (NASDAQ: HRMY)	ZYN002 CBD gel, Fragile X syndrome, applied to the skin (value driven by marketed sleep drug WAKIX)	Phase 3 missed primary endpoint Sep 2025	3,410.2
MapLight Therapeutics (NASDAQ: MPLT)	ML-004, autism social communication/irritability, oral (value driven by asset in schizophrenia)	Phase 2 missed primary endpoint Jun 2026	809.2
Charlotte's Web (TSX: CWEB)	AJA001 through ~one-third-owned DeFloria, irritability in autism, oral CBD extract	Phase 2-ready; initiation subject to funding	166.4

Source: company disclosures. Iress 23 September 2026

The Path from Here

Dosing the first patient confirms that Beyond Harmony is under way. The important test is whether the larger, multi-site trial can repeat HARMONY's encouraging result on Vineland-3, its measure of children's everyday functioning. A positive result would give Neurotech evidence to take into regulatory discussions and strengthen its position with potential partners; a negative result would cast doubt on its lead autism program. From here, recruitment pace will determine when that answer arrives, while progress on the planned US study and partnering discussions will show how Neurotech is preparing for the next stage.

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