

## Neurotech Files for US Orphan Drug Designation in Rett Syndrome

**Neurotech International Limited (ASX: NTI)** ('Neurotech', 'NTI' or 'the Company') a clinical-stage biopharmaceutical development company focused predominately on paediatric neurological disorders, has today submitted a request with the US Food and Drug Administration (FDA) for orphan drug designation (ODD) for the use of NTI164 in children and adults diagnosed with Rett Syndrome.

The FDA has authority to grant orphan drug designation to a drug or biological product to prevent, diagnose or treat a rare disease or condition. ODD qualifies sponsors for incentives including: (a) tax credits for qualified clinical trials (b) exemption from user fees and (c) potential seven years of market exclusivity after approval. The Orphan Drug Act defines a rare disease as a disease or condition that affects less than 200,000 people in the United States.

Rett Syndrome is a 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, which is required for normal brain development and function. Rett Syndrome occurs almost exclusively in girls, with incidence of one in 10,000 female live births. The prevalence is approximately 15,000 girls and women in the US and 350,000 globally. The market is estimated at over US\$2 billion annually.

A response from the FDA Office of Orphan Products Development is anticipated within 90 days of filing.

### Authority

This announcement has been authorised for release by the Board of Neurotech International Limited.

### Further Information

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### About Neurotech

**Neurotech International Limited (ASX:NTI)** is a clinical-stage biopharmaceutical development company focused predominately on paediatric neurological disorders with a broad-spectrum oral cannabinoid drug therapy called NTI164. Neurotech has completed a Phase II/III randomised, double-blind, placebo-controlled clinical trial in Autism Spectrum Disorder (ASD) with clinically meaningful and statistically significant benefits reported across a number of clinically-validated measures and excellent safety. In addition, Neurotech has completed and reported statistically significant and clinically meaningful Phase I/II trials in ASD and Paediatric Autoimmune Neuropsychiatric Disorders Associated with Streptococcal Infections (PANDAS) and Paediatric Acute-Onset Neuropsychiatric Syndrome (PANS), collectively PANDAS/PANS along with Rett Syndrome. Neurotech has received human ethics committee clearance for a Phase I/II clinical trial in spastic cerebral palsy.

For more information about Neurotech please visit <http://www.neurotechinternational.com>.

### **About NTI164**

NTI164 is a proprietary drug formulation derived from a unique cannabis strain with low THC ( $M < 0.3\%$ ) and a novel combination of cannabinoids including CBDA, CBC, CBDP, CBDB and CBN. NTI164 has been exclusively licenced for neurological applications globally. Pre-clinical studies have demonstrated a potent anti-proliferative, anti-oxidative, anti-inflammatory and neuro-protective effects in human neuronal and microglial cells. NTI164 is being developed as a therapeutic drug product for a range of neurological disorders in children where neuroinflammation is involved.

### **About Rett Syndrome**

Rett Syndrome is a 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, which is required for normal brain development and function. Rett Syndrome occurs almost exclusively in girls compared to boys (mostly fatal within one year of birth), with incidence of approximately 1 in 10,000 female live births across all racial and ethnic groups worldwide. According to the Rett Syndrome Research Trust, the prevalence is approximately 15,000 girls and women in the US and 350,000 globally.

Rett syndrome is characterized by typical early normal development between 7-18 months after birth, followed by a slowing of development, loss of functional use of the hands, distinctive hand movements along with difficulty walking, communicating, irritability and seizures. There is currently no cure for Rett Syndrome and one approved therapy in the United States. Current treatments only address symptoms and provide support that may improve movement, communication and social participation into adulthood.