Factsheet: Rett syndrome

BACKGROUND

Rett syndrome ('Rett') is a rare neurological condition and is classified as an autism spectrum disorder (ASD). Primarily affecting females and rarely found in males, it is a neurodegenerative disorder which means that, despite seemingly normal motor and cognitive development for the first 6-18 months of an infant's life, the child regresses considerably and loses the motor, cognitive and communications skills developed which affects all body movement. Rett syndrome is an orphan disease with no cure and an annual market opportunity estimated at over US$2 billion.

In most cases, Rett syndrome is caused by a mutation in the MeCP2 gene found on the X chromosome. A mutation in this gene disrupts the normal function of cells in the brain syndrome. Rett syndrome doesn't usually run through the family.

SYMPTOMS

Rett syndrome symptoms often present in stages.

- **Stage 1, early onset***:* generally occurs between 6 and 18 months of age. The child may be delayed in sitting, crawling, and making eye contact.
- **Stage 2, rapid destructive/regression stage***:* generally occurs between ages 1 to 4 and can take a few weeks or months. The child loses many of the skills developed including the use of hands, verbal communication, and walking. The child may experience problems with balance and coordination, have delayed head growth, difficulties with breathing, and may also experience strong emotional responses such as panic attacks.
- **Stage 3, plateau stage:** generally occurs between 2 and 10 years and is a phase of relative stability. The intensity of the symptoms from previous stages may lessen and the child can stay in this stage for years.
- **Stage 4, late motor deterioration:** generally occurs over the age of 10 and the child can experience a worsening of symptoms that last for many years or even decades including losing the ability to walk, muscle weakness, stiff or rigid muscles and joints, and general deterioration of the ability to move. Not all children will reach stage 4.

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DIAGNOSIS

Rett syndrome is diagnosed through assessments of a child's development, the physical signs and symptoms. Genetic testing is undertaken to confirm if there have been any changes to the MECP2 gene.

TREATMENT

There is currently no cure for Rett syndrome but there are treatments to reduce the impacts of the symptoms associated with the disorder. Occupational and physical therapies can help to support the child to maintain mobility or the purposeful use of the hands. Medical care can help to monitor and reduce symptoms such as muscle stiffness or seizures. There is also a new medication that has recently been approved by the U.S Food & Drug Administration (FDA) for use in the United States of America (USA) for children and adults with Rett syndrome. This has shown some improvement in symptoms for some patients.

NEUROTECH'S WORLD FIRST HUMAN CLINICAL STUDY TO TREAT RETT SYNDROME

Neurotech is conducting the world's first human clinical study to treat children with Rett syndrome. NTIRTT1 is a single-arm, open-label, Phase I/II clinical trial that will recruit 14 paediatric patients with a clinical diagnosis of Rett syndrome to determine the efficacy and safety of orally administered NTI164, a medicinal cannabis derived biopharmaceutical.

The trial is running at the Children's Hospital at Westmead, Sydney Australia and is being conducted under the guidance of Associate Professor Carolyn Ellaway, Clinical Geneticist in Metabolic Genetics at the New South Wales Genetic Metabolic Disorders Service, part of the Sydney Children's Hospital Network. Associate Professor Carolyn Ellaway is a widely renowned expert on Rett syndrome, having developed a widely used Rett questionnaire in 2001. Neurotech anticipates the top-line results of the NTIRTT1 trial will be available in the second half of 2023.

WHY NTI164?

Neuro-Inflammation is a common central mechanism for developmental cognitive impairment in Rett syndrome. Studies have confirmed that the mutation that causes Rett syndrome does cause uncontrolled inflammation throughout the body and the brain.

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Preclinical studies to date have shown that NTI164 is a potent anti-inflammatory, neuro-modulatory agent, making it an ideal candidate for the treatment of chronic neuro-inflammatory disorders, such as Rett syndrome.

NTI164's unique combination of CBDA and other minor cannabinoids work together to create an 'entourage effect' that is more potent than CBD alone, with only 0.3% THC. Preclinical and clinical studies to date have shown that NTI164 is a powerful neuro-anti-inflammatory modulator, can suppress a wide range of inflammatory cytokines, and improves neuronal cell viability and overall health.

FOR MORE INFORMATION

Visit https://neurotechinternational.com/biopharmaceutical-trials for up to date information about NTI164 and associated clinical trials focused on the treatment of rare paediatric neurological disorders including Autism spectrum disorder (ASD), PANS/PANDAS[1], Rett syndrome and cerebral palsy. NTI164 has been exclusively licenced by Neurotech International for neurological applications globally.

REFERENCES

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

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