**Trofinetide Improves Symptoms in Children and Adolescents With Rett Syndrome**

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By Andrew D. Bowser

WASHINGTON, DC -- October 30, 2017 -- The investigational agent trofinetide is safe and effective for children with Rett syndrome, researchers reported here at the 2017 Annual Meeting of the American Academy of Child and Adolescent Psychiatry (AACAP).

For children with this rare genetic disorder, “trofinetide is promising in not only ameliorating behaviours, but also a wide range of symptoms,” said Walter E. Kaufmann, MD, Greenwood Genetic Center, Greenwood, South Carolina.

This is the second phase 2 trial demonstrating the potential benefit of trofinetide in patients with Rett syndrome. The first was in adult patients, so the agent “has the potential to be beneficial across a variety of domains in Rett Syndrome, and potentially for other neurodevelopmental disorders,” said Dr. Kaufmann.

There are currently no approved treatments for Rett syndrome.

For the current phase 2 study, researchers randomised 82 patients aged 5 to 15 years to placebo or trofinetide 50, 100, or 200 mg/kg twice daily for 42 days.

The highest trofinetide dose demonstrated significant clinical benefit over placebo in several key measures, including the Rett Syndrome Behaviour Questionnaire total score, which focuses on core RTT symptoms (P = .042), the Clinical Global Impression-Global Improvement (P = .029), and RTT Domain-Specific Visual Analog Scale (P = .025), which measures the “most concerning” aspects of RTT as identified by clinicians.

Trofinetide was well tolerated and there no serious adverse events attributable to the drug. There were no patterns of adverse events evident with initiation or cessation of treatment.

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