# Innovative Research in Neurological Diseases Highlighted for Rare Disease Day 2025

*Neurolixis is proud to showcase its groundbreaking research and development for rare neurological conditions in honor of Rare Disease Day, on February 28, 2025.*

Rare Disease Day Raises awareness and generates change for the 300 million people worldwide living with a rare disease.

PARK RIDGE, NJ, UNITED STATES, February 19, 2025 /EINPresswire.com/ -- □□□□ □□□□□□□□ □□□ serves as an opportunity to raise awareness about the challenges faced by individuals living with rare diseases which affect up to 300 million people worldwide. Neurolixis is pioneering cutting-edge therapies aimed at addressing the unmet medical needs of patients suffering from three debilitating rare diseases - Spinocerebellar Ataxia (SCA), Fragile X Syndrome (FXS), and Rett Syndrome.

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> No patient should be left behind. On Rare Disease Day, we reaffirm our commitment to pioneering research that will improve the lives of those affected by rare neurological disorders.”
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> *Dr. Adrian Newman-Tancredi (CEO, Neurolixis)*

Spinocerebellar Ataxia is a group of inherited neurodegenerative disorders that lead to loss of motor control, affecting coordination and balance. Neurolixis is developing □□□-□□□ to alleviate motor symptoms, protect neurons, and slow the progression of the disease. Preclinical and early clinical results have shown promising improvements in motor function and stabilization of disease symptoms, offering new hope for individuals living with SCA.

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Fragile X Syndrome is the most common genetic cause of autism and intellectual disabilities. Neurolixis is developing □□□-□□□, a drug candidate that improves mood and cognition and shows disease-modifying neuroplastic activity in transgenic FXS mice. This suggests that it may alleviate core symptoms and enhance cognitive development

in children affected by Fragile X Syndrome.

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Rett Syndrome primarily affects young girls and leads to severe cognitive and motor impairments. The Neurolixis drug candidate, □□□-□□□, shows promising activity in transgenic Rett mice, improving their cognition and respiratory function and offering a potential path to clinical benefit for individuals living with Rett syndrome.

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Neurolixis specializes in the discovery and development of novel therapies for rare and neurological diseases. The Neurolixis drug candidates selectively target specific brain regions to modulate serotonin neuron activity. This approach aims to enhance therapeutic efficacy while minimizing side effects. The company's pipeline includes NLX-112 (Phase 2), which targets motor disorders, NLX-101 (Phase 1) for treating rare autism spectrum disorders, including Fragile X and Rett syndromes, and NLX-204, a preclinical candidate that shows potential as a rapid-acting antidepressant and analgesic, utilizing a non-opioid mechanism.

□ Information on Rare Disease Day: https://www.rarediseaseday.org/
□ 3-minute video summary of the Neurolixis rare disease programs: https://t.ly/SNq4H
□ Pharmacology review of Neurolixis serotonin 'biased agonists': https://t.ly/hCB8y

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