

## Neurolixis Awarded Grant from the International Rett Syndrome Foundation

### --Novel Approach to Treating Breathing Disorders in Rett Syndrome--

**Dana Point, 9 April, 2014** -- Neurolixis Inc. announced that it had been awarded a research grant by the International Rett Syndrome Foundation (IRSF) to test its clinical candidate, NLX-101, in animal models of Rett syndrome.

With this grant, Neurolixis will collaborate with researchers from the University of Bristol, U.K., led by Prof. Julian Paton (Principal Investigator) and Dr. Ana Abdala, who will evaluate the ability of the novel serotonin 5-HT1A 'biased agonist', NLX-101, to normalize abnormal cardiorespiratory functions and behaviors in transgenic mouse models of Rett syndrome.

Rett syndrome is a rare neurological disorder, affecting 1 in 10,000 female births. The syndrome only manifests itself when children are 1-2 years of age: girls then develop autistic-like behaviors, lose control of their muscles, lose speech and stop walking. Most children with Rett syndrome go on to develop breathing irregularities, epilepsy, severe learning disability, scoliosis and deformity in their extremities due to abnormal muscle control, among other health problems.

Notably, girls with Rett syndrome often experience spells of severe breath holding. These can cause decreases in blood oxygen levels that affect the brain, initiating or aggravating other symptoms such as epilepsy.

The Cure Rett Board of Trustees & Directors partnered with IRSF to share the cost of this grant.

Dr. Adrian-Newman Tancredi, Chief Scientific Officer of Neurolixis and Co-Principal Investigator, commented, "We thank the IRSF and Cure Rett for their financial support to this program, and are delighted to collaborate with the research team at the University of Bristol to replicate and extend our previous positive findings in Rett mice with NLX-101. New treatments such as NLX-101 need to be rigorously tested in animal models before moving to the clinic."

#### **About NLX-101**

NLX-101 acts on the brain's serotonin system, and exhibits preferential activation of 5-HT1A receptors located in specific regions of the brain: such 'biased agonism' in these brain regions is thought to underlie its potent effects in animal models of Rett syndrome (see Levitt ES et al., Journal of Applied Physiology, Oct 10, 2013). NLX-101 is an orally administered agent that has previously been tested in a Phase 1 clinical study in healthy volunteers. Neurolixis plans to investigate its ability to normalize irregular breathing patterns and other functions in patients with Rett syndrome.

#### **About Rett Syndrome**

Rett syndrome is a rare genetic postnatal neurological disorder that affects brain development, and primarily affects girls. Most babies with Rett syndrome develop normally at first, but symptoms appear between 6 and 18 months of age. Children with Rett syndrome develop a wide range of symptoms that include abnormal and distressing breathing patterns, as well as loss of speech and poor movement coordination. There is no cure for Rett syndrome. The incidence of Rett is estimated at 1 in 10,000 females; in the US & Europe, approximately 30,000 girls and women are affected with Rett syndrome.

### **About the International Rett Syndrome Foundation**

IRSF is the leading private funder of Rett syndrome research and a provider of family support programs to those touched by Rett syndrome. Through partnerships with local, national, and international supporters, IRSF has invested more than \$32 million in research leading to discoveries that allow testing of treatments for Rett syndrome in human clinical trials. IRSF has earned Charity Navigator's most prestigious 4 star rating. To learn more about IRSF and Rett syndrome, visit [www.rettsyndrome.org](http://www.rettsyndrome.org) or call IRSF at 1-800-818-RETT (7388).

### **About Neurolix, Inc.**

Neurolix, located in Dana Point, California, is a privately held biotechnology company developing therapies for disorders of the nervous system. The Company is focused on developing small molecule drugs for the treatment of neurological disorders such as Rett syndrome and Parkinson's disease, and psychiatric disorders such as depression and schizophrenia. Additional information regarding Neurolix is available at <http://www.neurolix.com>.

### **Forward Looking Statement**

Except for the historical information contained herein, the matters discussed in this press release are forward-looking statements that involve risks and uncertainties, including: our dependence on third parties for the development, regulatory approval and successful commercialization of our products, the inherent risk of failure in developing product candidates based on new technologies, risks associated with the costs of clinical development efforts, as well as other risks. Actual results may differ materially from those projected. These forward-looking statements represent our judgment as of the date of the release. Neurolix disclaims any intent or obligation to update these forward-looking statements.

### **PRESS CONTACTS**

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