

## Neurolix Awarded Grant from the Rett Syndrome Research Trust

**Dana Point, 24 June, 2015** -- Neurolix Inc. announced that it had been awarded a second research grant by the Rett Syndrome Research Trust (RSRT) to support the development of its novel serotonin 5-HT1A receptor agonist, NLX-101. This award will help support Chemistry, Manufacturing, and Controls (CMC), preparation of clinical protocols and filing of an Investigational New Drug (IND) Application in support of a clinical study with NLX-101 in Rett syndrome patients.

Dr. Mark Varney, Chief Executive Officer of Neurolix commented, "We greatly appreciate the RSRT's financial support for this program. Previous work carried out with RSRT support helped us investigate the pharmacokinetics of NLX-101 in preparation for a future clinical study. The new grant will now enable us to move the program through the regulatory steps necessary to test NLX-101 in Rett syndrome patients."

Monica Coenraads, co-founder and Executive Director of the RSRT and the mother of a teenaged daughter with the disorder, commented: "NLX-101 has a profile of activity that distinguishes it from other drugs currently being investigated for Rett syndrome. We are pleased to work with Neurolix in advancing this original program and eager to see the drug tested in the clinic."

Dr. Varney added: "Rett syndrome is a devastating disorder and we believe that NLX-101 has the potential to alleviate some of the symptoms that negatively affect the quality of life of the patients."

### About NLX-101

NLX-101 is a small molecule pharmacotherapeutic that selectively targets serotonin 5-HT1A located in specific regions of the brain controlling respiration, mood and cognition. Such 'biased agonism' is thought to underlie its potent effects in animal models of Rett syndrome (see Levitt ES et al. Journal of Applied Physiology, 2013; Abdala AP et al. Frontiers in Physiology, 2014). NLX-101 is an orally administered agent that has previously been tested in a Phase 1 clinical study in healthy volunteers. NLX-101 has been awarded Orphan Drug designation in both the USA and the European Union. Neurolix plans to investigate the ability of NLX-101 to normalize irregular breathing patterns and other functions in patients with Rett syndrome.

### About Rett Syndrome

Rett syndrome is a rare childhood neurological disorder that primarily affects girls. Most babies with Rett syndrome develop normally at first, but symptoms appear between 6 and 18 months of age and may include abnormal and distressing breathing patterns, loss of speech, abnormal muscle control that often affects the ability to walk, epilepsy, severe learning disability, scoliosis, anxiety and gastrointestinal problems. There is no cure for Rett syndrome. The incidence of Rett is estimated at 1 in 10,000 females; worldwide approximately 350,000 girls and women are affected with Rett syndrome.

## **About the Rett Syndrome Research Trust**

The Rett Syndrome Research Trust (RSRT) is a non-profit organization with a highly focused and urgent goal: to drive the development of treatments and cures for Rett Syndrome and related *MECP2* disorders. RSRT operates at the center of global scientific activity, leading and advancing the research agenda. To learn more please visit [www.rsrt.org](http://www.rsrt.org).

## **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 [www.neurolix.com](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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