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**NEUROLIXIS ANNOUNCES FDA APPROVAL OF ORPHAN DRUG STATUS FOR NLX-101 IN THE TREATMENT OF RETT SYNDROME**

San Diego, CA, November 5, 2013 -- Neurolix, Inc., a biopharmaceutical company that discovers and develops novel treatments to treat disorders of the brain, today announced that NLX-101, its selective serotonin 5-HT<sub>1A</sub> receptor 'biased agonist' has received Orphan Drug designation from the U.S. Food and Drug Administration (FDA) for the treatment of Rett syndrome. Rett syndrome is a serious neurological disorder that causes problems in many brain functions and a wide range of disabilities.

"The FDA's orphan drug designation of NLX-101 is an important milestone for Neurolix, as we embark on the development work required to initiate clinical studies in Rett patients," commented Mark A. Varney, Ph.D., CEO of Neurolix, Inc.

Orphan drug designation is granted by the FDA Office of Orphan Drug Products to novel drugs that treat a rare disease affecting fewer than 200,000 patients in the U.S. The designation provides the drug developer with a seven-year period of U.S. marketing exclusivity, as well as tax credits for clinical research costs, the potential to apply for annual grant funding, clinical research trial design assistance and a waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

**About NLX-101**

Neurolix in-licensed NLX-101 (also known as F-15599) from Pierre Fabre Medicament. NLX-101 has previously been tested in Phase 1 clinical studies in healthy volunteers, and Neurolix plans to investigate its ability to normalize irregular breathing patterns and other functions in patients with Rett syndrome. In preclinical studies, NLX-101 reversed breathing deficits in transgenic mice with the MeCP2 gene mutation that underlies Rett's syndrome. These data are described in a peer-reviewed manuscript recently published (*Levitt ES et al., Journal of Applied Physiology, Oct 10, 2013*).

NLX-101 acts on the brain's serotonin system, and exhibits preferential activation of frontal cortex-located 5-HT<sub>1A</sub> receptors: such 'biased agonism' in this brain region is thought to underlie its potent antidepressant-like activity and its capacity to

attenuate cognitive deficits in rodent models. These properties are relevant to patients suffering from Rett syndrome, which is also associated with mood deficits and cognitive dysfunction.

### **About Rett Syndrome**

Rett syndrome is a rare neurodevelopmental disorder that affects brain development, and primarily affects girls. Most babies with Rett syndrome to 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 United States approximately 16,000 girls and women affected.

### **About Neurolixis, Inc.**

Neurolixis, located in San Diego, 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 psychiatric disorders such as depression and schizophrenia, and neurological disorders such as Parkinson's disease and Rett syndrome. Additional information regarding Neurolixis is available at <http://www.neurolixis.com>.