

***Company Contact:***

Mark A. Varney, PhD  
President and CEO  
Neurolix, Inc.  
(+1) 949.214.4017

**Neurolix Receives Orphan Medicinal Product Designation In The European Union For A Novel Therapy To Treat Rett Syndrome**

San Diego, CA, March 6, 2014 -- 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 been granted Orphan Medicinal Product Designation from the European Commission for the treatment of Rett syndrome. This decision follows a positive opinion adopted by the Committee for Orphan Medicinal Products (COMP), recommending such designation. Rett syndrome is a rare genetic neurologic condition affecting mostly girls, causing problems in many brain functions resulting in a wide range of disabilities.

Orphan Medicinal Product designation provides 10 years of market exclusivity upon receipt of European Union marketing approval. This period of marketing exclusivity can be extended by two additional years for medicines that have also complied with an agreed pediatric investigation plan, which Neurolix intends to submit. It also allows for regulatory assistance in preparing the marketing application, free protocol assistance to optimize clinical development, reduced regulatory fees associated with applying for marketing approval and direct access to the centralized procedure for Marketing Authorization Application through the European Medicines Agency. This allows companies to make a single application and receive a single opinion and decision from the European Commission, valid in all EU member states.

"We are pleased to receive orphan designation of NLX-101 from the European Commission. It 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.

**About NLX-101**

NLX-101 acts on the brain's serotonin system, and exhibits preferential activation of 5-HT<sub>1A</sub> 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 neurodevelopmental 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 European Union approximately 16,000 girls and women affected with a similar number in the United States.

### **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>.

### **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. Neurolixis disclaims any intent or obligation to update these forward-looking statements.