Neurolixis Secures Orphan Medicinal Product Designation for NLX-112 as a Treatment for Spinocerebellar Ataxia

European Commission approves OMP Designation for NLX-112 (befiradol) following compelling results in studies funded by the US Dept of Defense.

CASTRES, FRANCE, September 25, 2024 /EINPresswire.com/ -- Neurolixis Inc., a biopharmaceutical company pioneering innovative therapies for brain disorders, announced that NLX-112, a highly selective serotonin 5-HT1A receptor agonist, has been granted Orphan Medicinal Product (OMP) Designation by the European Commission for the treatment of Spinocerebellar Ataxia (SCA; approval #EU/3/24/2951). This decision follows a positive recommendation from the Committee for Orphan Medicinal Products (COMP).

> This OMP designation is a milestone for Neurolixis as we expand our programs targeting rare neurological disorders. With NLX-112, we are making strides in the field of movement disorders."
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> *Dr. Adrian Newman-Tancredi, CEO of Neurolixis*

OMP designation offers several significant benefits, including ten years of market exclusivity following European Union marketing approval. An additional two years of exclusivity may be granted if Neurolixis successfully complies with a pediatric investigation plan, which the company intends to submit.

Spinocerebellar Ataxia is a group of rare genetic disorders, most notably Machado-Joseph Disease (SCA3), which causes progressively debilitating neurological symptoms such as clumsiness, muscle weakness, and tremors. Without effective treatment, patients ultimately experience severe disability.

The OMP designation for NLX-112 was granted based on a successful collaboration between Neurolixis and Professor Patricia Maciel's team at the University of Minho in Portugal. This partnership, funded by the U.S. Department of Defense, demonstrated that NLX-112 significantly reduces motor dysfunction in preclinical models of SCA3.

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NLX-112 (also known as befiradol) is an oral drug that selectively activates serotonin 5 HT1A receptors, demonstrating significant efficacy in preclinical models of motor disorders. The compound has shown positive results in a clinical safety, tolerability, and efficacy Phase 2A trial in Parkinson’s disease (2023). NLX-112 has been safely tested in more than 600 patients for various non-motor indications, further supporting its potential in neurological treatments.

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Machado-Joseph Disease (SCA3) is a rare, inherited motor disorder marked by symptoms such as clumsiness, difficulty with speech and swallowing, and a staggering gait. In some cases, patients may also develop dystonia or symptoms resembling Parkinson's disease. The disease often begins in adolescence, worsening over time and eventually leading to paralysis. There is currently no cure or approved treatment for SCA3.

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Neurolixis is a privately held biotechnology company focused on developing innovative therapies for neurological disorders. In addition to NLX-112, which targets motor disorders, Neurolixis is advancing NLX-101 (Phase 1) for treating rare autism spectrum disorders, including Fragile X and Rett syndromes. The company is also developing NLX-204, a preclinical candidate that shows potential as a rapid-acting antidepressant and analgesic, utilizing a non-opioid mechanism.
For more information about Neurolixis, please visit www.neurolixis.com.

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The mission of University of Minho, founded in 1973, is to create, spread and convert knowledge into value-generating applications. The Life and Health Sciences Research Institute (ICVS) is the Biomedicine R&D Unit of the School of Medicine cluster of the University. The cluster includes 2CA-Braga, a clinical research site that currently runs 1/3 of all clinical trials in Portugal; P5, that with the ICVS conceives and develops new digital medicine products and provides medical services with a digital interface; and B.ACIS, a knowledge transfer interface fostering developments in biomedical research resulting in marketed products that bring clinical benefits to patients.

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This press release may contain forward-looking statements, including Neurolixis' dependence on third-party collaborations, regulatory approval, and commercialization efforts. These statements involve risks and uncertainties, and actual results may vary. Neurolixis undertakes no obligation to update these forward-looking statements.

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