# Newron Pharmaceuticals Announces Expansion of STARS Study to Include Patients Under 13 Years of Age

## Recruitment for potentially pivotal study in patients suffering from Rett syndrome ongoing in the USA, Europe, Asia and Australia

**Milan, Italy and Morristown, N.J., USA – May 17, 2017 –** Newron Pharmaceuticals S.p.A. ("Newron") (SIX: NWRN), a biopharmaceutical company focused on the development of novel therapies for patients with diseases of the central nervous system (CNS) and pain, today announced that the Sarizotan Treatment of Apneas in Rett Syndrome (STARS) study has been expanded to include Rett syndrome patients as young as six years of age. An amendment to allow the inclusion of the patients under the age of 13 was approved by the FDA after Newron submitted results from juvenile toxicology studies. Newron is also submitting the amendment to Health Authorities and Institutional Review Boards in other countries.

Ravi Anand, M.D., Chief Medical Officer at Newron, commented: "Rett syndrome is a devastating condition that manifests in early childhood. This decision by the FDA to lower the age limit for inclusion of patients from six years old will allow sarizotan's benefits to be evaluated in these younger patients before the disease has significantly progressed. Newron believes that earlier onset of treatment in Rett's patients may be associated with less deterioration of respiratory and neurological symptoms."

STARS is a randomized, double blind, placebo-controlled study, expected to enroll 129 patients (three groups of 43), in up to 15 centers of excellence in the USA, the UK, Italy, Australia and India. The potentially pivotal clinical study will evaluate the efficacy, safety and tolerability of sarizotan in patients with Rett syndrome suffering from respiratory symptoms. Among the core symptoms of Rett, breathing disturbances may affect the whole body; they can have a marked effect on biochemistry, influence emotions, circulation and digestive function as well as musculoskeletal structures in the respiratory process.

The primary endpoint for the STARS study is reduction in episodes of clinically significant apneas (>10 sec) during waking time by 20 percent. During this 6-month study, patients will receive treatment with daily doses of 10 and 20 mg of sarizotan, or placebo. The Company expects to have top-line results available for release in 2018.

Sarizotan received Orphan Drug Designation for the treatment of Rett syndrome from both the European Commission and the FDA in 2015. It could become the first therapy approved for the treatment of Rett syndrome patients. Newron intends to commercialize sarizotan directly.

Besides the STARS efficacy study, Newron, as part of its commitment to the rare disease patient community, is partnering with the global Rett community to work on the first Burden of Disease (BOD) study. The study aims to deliver data and analytics to quantify the physical, emotional and financial challenges of Rett syndrome. These learnings can help identify improved intervention programs and services designed to complement the Rett care pathway.

**Learn More**
View a video on Rett syndrome here.

**About Rett Syndrome**

Rett syndrome is a severe neurodevelopmental disorder primarily affecting females, with an estimated prevalence ranging from one in 10,000 to one in 20,000 females. There are no approved treatments available. Rett syndrome is characterized by a loss of acquired fine and gross motor skills and the development of neurological, cognitive and autonomic dysfunction, which leads to loss of ability to conduct daily life activities, walk or communicate. Rett syndrome also is associated with a reduced life expectancy. Approximately 25 percent of the deaths in patients with Rett syndrome are possibly related to multiple cardio-respiratory dysrhythmias that result from brain stem immaturity and autonomic failure. More than 95 percent of these patients have a random mutation in the MECP2 gene. Episodes of apnea, hyperventilation and disordered breathing are found in approximately 70 percent of patients with Rett syndrome at some stage of their life. For more information on Rett syndrome, visit www.rettsyndrome.org.

**About Newron Pharmaceuticals**

Newron (SIX: NWRN) is a biopharmaceutical company focused on the development of novel therapies for patients with diseases of the Central Nervous System (CNS) and pain. The Company is headquartered in Bresso near Milan, Italy. Xadago® (safinamide) has received marketing authorization for the treatment of Parkinson's disease in the European Union, Switzerland and the USA, and is commercialized by Newron's Partner Zambon. US WorldMeds holds the commercialization rights in the USA. Meiji Seika has the rights to develop and commercialize the compound in Japan and other key Asian territories. In addition to Xadago® for Parkinson's disease, Newron has a strong pipeline of promising treatments for rare disease patients at various stages of clinical development, including sarizotan for patients with Rett syndrome and ralfinamide for patients with specific rare pain indications. Newron is also developing Evenamide as the potential first add-on therapy for the treatment of patients with positive symptoms of schizophrenia. **www.newron.com**

For further information


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By their very nature, such statements and assumptions involve inherent risks and uncertainties, both general and specific, and risks exist that predictions, forecasts, projections and other outcomes described, assumed or implied therein will not be achieved. Future events and actual results could differ materially from those set out in, contemplated by or underlying the forward-looking statements due to a number of important factors. These factors include (without limitation) (1) uncertainties in the discovery, development or marketing of products, including without limitation negative results of clinical trials or research projects or unexpected side effects, (2) delay or inability in obtaining regulatory approvals or bringing products to market, (3) future market acceptance of products, (4) loss of or inability to obtain adequate protection for intellectual property rights, (5) inability to raise additional funds, (6) success of existing and entry into future collaborations and licensing agreements, (7) litigation, (8) loss of key executive or other employees, (9) adverse publicity and news coverage, and (10) competition, regulatory, legislative and judicial developments or changes in market and/or overall economic conditions.

Newron may not actually achieve the plans, intentions or expectations disclosed in forward-looking statements and assumptions underlying any such statements may prove wrong. Investors should therefore not place undue reliance on them. There can be no assurance that actual results of Newron's research programmes, development activities, commercialisation plans, collaborations and operations will not differ materially from the expectations set out in such forward-looking statements or underlying assumptions.

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