Newron provides update on STARS clinical study with sarizotan for the treatment of Rett syndrome

- ***Trial now expected to complete enrollment in H2 2018, with data readout expected in H1 2019***
- ***90% of patients have continued into a long-term extension study***
- ***Rett syndrome is an orphan disease with no approved treatment***

Milan, Italy and Morristown, NJ, USA - June 13, 2018 - 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 and peripheral nervous system, today announces that the STARS clinical study (Sarizotan Treatment of Apneas in Rett Syndrome) in patients with Rett syndrome is expected to complete enrollment in H2 2018. Newron expects to report results from the STARS study in H1 2019.

Currently over 100 patients, aged six years and over, have qualified for inclusion into the trial. Based on the current rate of screening and qualification, Newron anticipates that randomization of the target number of 129 patients will be completed in H2 2018. The STARS study is being performed in patients who present with clinically significant apneas during the course of the disease. Apneas are a cardinal feature of Rett syndrome, present in approximately 70% of patients, and contribute significantly to other co-morbidities, as well as leading to a reduced quality of life. Rett syndrome, a severe neuro-developmental orphan disease with no approved treatment options, overwhelmingly affects girls starting at a very young age.

Only patients who experience at least 10 episodes per hour of clinically significant apnea, (i.e. more than 10 seconds each in duration, while awake during the day) qualify for inclusion in the study. These recordings are performed over a 5-6-hour period per day, over 3 days per week, with the opportunity for the patients to repeat the procedure in the subsequent 3 weeks in case they do not qualify in the first week of screening. Patients who meet eligibility criteria are randomized to treatment or placebo and undergo the same recording of respiration at home at four separate time-points during the 24-week double-blind period of the study. The primary endpoint for the STARS study is reduction in episodes of these apneas 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.

Treatment with sarizotan has been very well tolerated, with a very low rate of discontinuation due to adverse events or lack of efficacy. Approximately 90 percent of the patients who have completed the 24-week double-blind period have continued in the long-term open-label extension. The safety of the patients in the trial is overseen by an independent international safety monitoring board which has reviewed all safety data and has recommended that the study be continued without any modification.

About Rett Syndrome
Rett syndrome is a severe neurodevelopmental disorder primarily affecting females, with an estimated prevalence of one in 10,000 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 http://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 and peripheral nervous system. 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. For more information, please visit: **www.newron.com**

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