Newron Completes Patient Enrollment in Pivotal STARS Study

- ***Top line results expected in Q4 2019***
- ***Almost all patients continued treatment with sarizotan in a long-term extension study***
- ***Positive study results may lead to the approval of the first drug to benefit a key symptom of Rett syndrome, a severe neuro-developmental orphan disease***

Milan, Italy and Morristown, NJ, USA - February 1, 2019 - 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 announced the successful completion of patient enrollment in its STARS (Sarizotan for the Treatment of Apneas in Rett Syndrome) clinical study. Newron expects to report top line results from the STARS study in Q4 2019.

Rett syndrome, a severe neuro-developmental orphan disease with no approved treatment options, overwhelmingly affects girls starting at a very young age. Currently, more than 130 patients of four years or older, have qualified for inclusion into Newron's study, which is being conducted at 14 centers throughout the US, Europe, Asia and Australia. 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 percent of patients, which contribute significantly to other co-morbidities, as well as to a reduced quality of life.

Only patients who experienced clinically significant apnea, i.e. at least 10 episodes of more than 10 seconds each per hour while awake, qualified for inclusion in the study. The apneas were monitored and recorded using a state-of-the-art medical device providing an objective measure of breathing dysfunction. The recordings were performed over a 5- to 6-hour period per day, for 3 days per week, with the opportunity for patients to repeat the procedure in the subsequent 3 weeks in case they did not qualify in the first week of screening. During the 6-month study, patients who met the eligibility criteria were randomized for treatment with daily doses of 10 or 20 mg of sarizotan, or placebo. Recordings of respiration take place at home, at four separate time-points during the 24-week double-blind period of the study, which is still ongoing for the last patients enrolled. The primary endpoint of the STARS study is the percentage reduction in these episodes of apnea during waking time compared with placebo.

Treatment with sarizotan has been very well tolerated to date, 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 study. 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.

During its R&D day on October 31, 2018, Newron presented baseline data from more than 100 patients enrolled in the STARS study, for the first time providing an objective measure of breathing dysfunction in the home environment over a long period of wakefulness. Data suggests that untreated, up to 70 percent of patients experience clinically significant apnea and minimally 10 percent of their time is spent without breathing. In these patients, oxygen saturation falls below 90 percent between 4.2 to 24 times per hour, and the cumulative duration of this state may last as long as 48 minutes per hour.

Anticipated clinical results from the STARS study, if positive, could position the Company to submit a filing for marketing authorization with the US, Canadian and European regulatory agencies. The Company continues its plans to commercialize sarizotan in key territories and has been working with the Rett syndrome community to build disease awareness.

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, the USA, Australia and Canada, 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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