Newron Supports Global Rare Disease Day® 2019

Sponsoring international study to examine the burden of Rett syndrome on patients and caregivers, featured at Findacure's Drug Repurposing for Rare Diseases Conference in the UK

Milan, Italy and Morristown, NJ, USA, February 28, 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, is pleased to support this year's Rare Disease Day®. Observed today, February 28, Rare Disease Day helps to raise awareness of rare diseases, and in turn improve access to treatments.

As part of its commitment to the rare disease patient community, Newron has partnered with the global Rett community in conducting the ongoing, first International Burden of Disease study. Rett syndrome is a severe neuro-developmental orphan disease with no approved treatment options and overwhelmingly affects girls starting at a very young age. Apneas are an unfortunate feature of Rett syndrome, present in approximately 70 percent of patients, and contribute significantly to other co-morbidities as well as to a reduced quality of life. The study aims to deliver data and analytics to quantify the physical, emotional and financial challenges of Rett syndrome. The results will help identify and guide improved intervention programs and services designed to complement the Rett care pathway.

Newron is also strongly supportive of the role that drug repurposing can play in accelerating the development of rare disease treatments. Newron's sarizotan, a new chemical entity, which was initially studied in another indication, has since been repurposed and is currently being studied in the STARS (Sarizotan for the Treatment of Apneas in Rett Syndrome) clinical trial.

Findacure, a UK charity supporting the rare disease community to drive research and develop treatments, yesterday held the Drug Repurposing for Rare Diseases Conference in London, UK. At the conference, Rachael Stevenson, CEO of Reverse Rett, and Becky Jenner, CEO of Rett UK, two leading advocates for the Rett patient community, discussed the potential for improving the lives of children and adults with Rett syndrome in the UK and also shared their experiences of their daughters who participated in Newron's STARS trial in the UK.

Dr. Richard Thompson, CEO of Findacure, said, "Drug repurposing can make use of our existing knowledge about a drug and its interactions with humans to potentially accelerate the pace of drug discovery. There is an enormous unmet need, as more than 7,000 rare diseases have been identified but only 400 have an approved treatment. Effective drug repurposing can have significant potential to bring treatments to patients with little hope of ever receiving pharmaceutical intervention."

"Through our commitment to the Rett community, we work with caregivers, advocacy groups, physicians and government agencies to help deliver what patients need," said Dennis Dionne, Newron Pharmaceutical's Vice President of Commercial Affairs. "We are proud to engage in a meaningful way with Rare Disease Day, and to support the advocacy organizations that are helping to improve the quality of life for patients and their families."

About Rare Disease Day®
Rare Disease Day takes place on the last day of February each year, a month known as having a 'rare' number of days, thus on February 28, 2019. The main objective of Rare Disease Day is to raise awareness among the general public and decision-makers about rare diseases and their impact on patients' lives. The campaign targets primarily the general public and seeks to raise awareness among policy makers, public authorities, industry representatives, researchers, health professionals and anyone who has a genuine interest in rare diseases. The campaign started as a European event and has progressively become a world phenomenon, with the USA joining in 2009 and participants in over 90 countries throughout the world in both 2017 and 2018. On rarediseaseday.org you can find information about the thousands of events happening around the world on the last day of February.

About Rett Syndrome
Rett syndrome is a severe neuro-developmental 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.

STARS Study
Newron has successfully completed patient enrollment in the Sarizotan Treatment of Apneas in Rett Syndrome (STARS) study, a clinical study to 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 person body; they can have a marked effect on biochemistry, influence emotions, circulation and digestive function as well as musculoskeletal structures in the respiratory process.

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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