



Newron Supports Global Rare Disease Day® 2017 and Rett Syndrome Studies

Milan, Italy and Morristown, NJ, USA, February 21, 2017 - Newron Pharmaceuticals S.p.A. ("Newron"), a biopharmaceutical company focused on the development of novel therapies for patients with diseases of the central nervous system (CNS) and pain, today announced its support of this year's Rare Disease Day® - which takes place worldwide on the last day of February each year – and the Rett community to raise awareness of the importance of research for rare diseases. United together, patients and caregivers, researchers and advocacy groups are working in common to elevate understanding and improve the lives of those impacted by rare diseases.

"We are proud to continue our support of NORD (The National Organization for Rare Disorders in the USA) and EURORDIS (the European Organization for Rare Diseases) as they work to increase awareness for rare diseases and the unique challenges of those affected," said Dennis Dionne, Vice President Commercial Affairs, Newron. "We are particularly honored to support groups such as Rettsyndrome.org which is dedicated to improving the lives of girls living with Rett syndrome."

Burden of Disease Study

As part of its commitment to the rare disease patient community, Newron 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](#).

STARS Study

Newron has also initiated the Sarizotan Treatment of Apneas in Rett Syndrome (STARS) study, a potentially pivotal 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 Rare Disease Day®

Rare Disease Day takes place on the last day of February each year, thus on February 28, 2017. The main objective of Rare Disease Day is to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives. The campaign targets primarily the general public and also seeks to raise awareness amongst 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 80 countries throughout the world in 2016.



In the United States, the National Organization for Rare Disorders (NORD) promotes and celebrates Rare Disease Day. For more information about Rare Disease Day in the U.S., please visit <http://www.rarediseaseday.us>. For information about global activities, please visit www.rarediseaseday.org.

About Rare Diseases

The European Union considers a disease as rare when it affects fewer than 5 in 10,000 citizens. According to EURORDIS, over 6,000 different rare diseases have been identified to date, affecting over 60 million people in Europe and the USA alone. Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offering inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.

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 and Switzerland and is commercialized by Newron's partner Zambon. US WorldMeds holds the commercialization rights in the US. 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**



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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.

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Newron does not undertake any obligation to publicly update or revise forward-looking statements except as may be required by applicable regulations of the SIX Swiss Exchange, where the shares of Newron are listed.

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