



Newron Pharmaceuticals to Present at European Rett Syndrome Congress and Host Advisory Board Meeting

- **Continued advancement of the International Burden of Disease in Rett Syndrome Study**
- **Oral presentation on findings from concept elicitation interviews with caregivers and healthcare providers**

Milan, Italy & Morristown, N.J. - October 25, 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 and peripheral nervous system, today announced an oral presentation at the upcoming [European Rett Syndrome Congress](#) (“the congress”) in Berlin, Germany, November 2-4, 2017. At the congress, Newron will also host an advisory board meeting as part of the International Burden of Disease Study, evaluating the burden of disease experienced by patients with Rett syndrome and their families.

The advisory board meeting will focus on refining the development of two surveys, which will be used to examine the burden of Rett syndrome on individuals and their caregivers in Europe and the United States. The advisory panel comprises caregivers and a diverse variety of healthcare professionals within the international Rett syndrome community.

An oral presentation entitled “Important symptoms of Rett syndrome and their impact: the perspective of parents and healthcare providers” has been accepted by the congress for an oral presentation on Saturday, November 4, 9:30 -11:00 am CET. The presentation will include the results of interviews conducted among caregivers and healthcare providers.

“The Burden of Disease Study is the largest research study characterizing the burden and impact of Rett syndrome on patients and families. The study provides systematic information currently unavailable, and will help guide physicians, caregivers, and policy experts on the human and financial cost of this devastating disease”, said Walter E. Kaufmann, MD, [Greenwood Genetic Center](#); [rettsyndrome.org](#)’s Medical Advisory Board and Scientific Review Board member; and Chair of [RettSearch](#), the consortium of Rett clinical researchers. Dr. Kaufmann is scheduled to speak at the congress on the direction of future research projects and new treatment options for individuals living with Rett syndrome.

Leopold Curfs, Professor of Intellectual Disabilities, [Maastricht University Medical Centre](#); Director of the Rett Expertise Centre Netherlands-GKC, noted, “It is heartening to see the dedicated work researchers have done to improve the lives of girls living with Rett syndrome. We are optimistic about the Burden of Disease Study which may support the development of a standardized methodology to assist in the development and availability of new orphan treatments.”



Newron will also present a poster showing the rationale and design for the global, double-blind, placebo-controlled study STARS (Sarizotan Treatment of Apneas in Rett Syndrome). This study utilizes novel methodologies developed to assess respiration in the home environment of Rett syndrome patients who are experiencing severe apneic breathing while awake. Breathing irregularity is a day-to-day challenge that can also affect muscles, mood and metabolism.

“As a mother of a Rett patient, I know how one symptom can have a broader impact. Newron’s studies evaluating sarizotan in the treatment of apneas and burden of disease in Rett syndrome break new ground. It is comforting to see the search for treatments today that may improve the lives of patients with Rett syndrome and their families as we continue to await advances in gene therapy,” commented Orietta Mariotti, Adviser, [Pro Rett Ricerca Italy](#).

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 <http://www.newron.com/en>.

For more information, please contact

Newron

Stefan Weber – CEO
+39 02 6103 46 26
pr@newron.com

UK/Europe

Julia Phillips, FTI Consulting
+44 20 3727 1000
julia.phillips@FTIConsulting.com

**Switzerland**

Martin Meier-Pfister, IRF Communications
+41 43 244 81 40
<mailto:martin.meier-pfister@irfcom.ch>

Germany/Europe

Anne Hennecke, MC Services
+49 211 52925222
anne.hennecke@mc-services.eu

USA

Beth Kurth, LaVoieHealthScience
+1 617 374 8800, Ext. 106
bkurth@lavoiehealthscience.com

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