



## **Newron Pharmaceuticals Announces Advisory Board Recommendations and Rett Congress Findings**

- **Advisory Board Characterizes Illness and Treatment Patterns**
- **Rett Congress Reviews Multi-Organ Impact of Single Symptoms**

**Milan, Italy & Morristown, N.J.** – November 7, 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 recommendations from its advisory board, who are evaluating the burden of disease experienced by patients with Rett syndrome and their families, as well as an update from the recent [European Rett Syndrome Congress](#) in Berlin, Germany. Newron is currently developing sarizotan, the first new chemical entity (NCE) in a late Phase 3 clinical trial in Rett syndrome.

At the Rett Congress, Newron’s Burden of Disease advisory board reached a consensus on the concept elicitation interviews which are being used to develop a survey that will be distributed internationally to caregivers and allied healthcare professionals. The study assesses the impact of symptoms on daily activities while also capturing the frequency of clinical events and resource utilization as reported by caregivers for Rett syndrome patients.

At the advisory board meeting, all significant symptoms of Rett syndrome were reviewed including breathing abnormalities, mobility, communication, sleep disturbances, bone health, and other symptoms of this multi-organ disease. Speakers agreed that in Rett syndrome patients, one symptom can have a significant impact on other symptoms. Girish Sharma, MD, Pulmonologist, [Rush University Medical Center](#), commented, “Ongoing chronic respiratory abnormalities, including acute episodes such as hyperventilation, may result in biochemical and metabolic changes with an impact on multiple systems and stress levels in the patient and caregiver. We look forward to utilizing the Burden of Disease study to help understand gaps in managing a patient’s condition throughout the whole body.”

The well attended [European Rett Syndrome Congress](#) also included an oral presentation entitled “Important symptoms of Rett syndrome and their impact: the perspective of parents and healthcare providers.” During the podium session, Dr. Kate Williams, Senior Consultant, Clinical Outcomes Assessment at [PAREXEL International](#), presented an update on the findings from the concept elicitation interviews with caregivers of individuals with Rett syndrome and healthcare providers. The findings from the interviews demonstrated that some symptoms, for example respiratory abnormalities, impact other symptoms such as gastrointestinal gas and subsequent pain. Other symptoms, such as mobility and muscle tone, were found to impact each other bi-directionally.

Separately, Newron hosted a roundtable discussion on the need for new tools to assess the health economic value of orphan drugs treating rare disease that impact multiple organ systems. “Although only a small number of patients suffer from each rare disease, collectively rare diseases are a significant medical and social issue,” said Dennis Dionne, VP of Commercial Affairs at Newron. “The



advent of improved methods for assessing the value of orphan drugs will enable more and better therapies for rare diseases to be made available to patients.”

#### **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>.

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