



## **Newron to Evaluate Burden of Disease for Rett Syndrome Patients and Families**

**Milan, Italy and Morristown, N.J., USA – June 27, 2016** – 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 nervous system (CNS) and pain, today announced that it is sponsoring a study to evaluate the burden of disease experienced by patients with Rett syndrome and their families.

The announcement was made during the international 2016 Rettsyndrome.org Research Symposium, held in Itasca, Illinois, USA, from June 22–24, 2016. The expert advisory panel governing the study is comprised of preeminent Rett syndrome specialists, Rettsyndrome.org advocacy leaders and patient families.

“This will be the first international study to characterize the disease, including disease - related quality of life, frequency of clinical events and resource utilization that will evaluate the burden and impact of Rett Syndrome on patients and families. The study will provide detailed information currently unavailable to help guide physicians, caregivers and policy experts about the human and financial cost of this devastating disease,” said Alan K. Percy, M.D., Professor of Pediatric Neurology, Department of Neurobiology at the University of Alabama (UAB) School of Medicine, Birmingham, Alabama, USA., Director of the UAB Sparks Clinics Rett Syndrome Research Program and former Director of the American Board of Psychiatry and Neurology.

Rett syndrome is characterised by severe neurological, respiratory, cognitive, and behavioral symptoms. The rare disease, primarily affecting approximately one in 10,000 females, leads to severe physical and psychological disabilities and many patients require lifelong 24-hour care. However, the impact of the disease on the amount and quality of care required for the management of these patients and the burden of care imposed on the families is currently unknown.

The study will be comprised of two global surveys, one to be completed by 500 caregivers and the other by 50 physicians. Both will examine patient burden, with the caregiver survey additionally evaluating caregiver burden. The surveys are being developed in accordance with regulatory guidance, with the final versions being used for data collection in the United States, the United Kingdom, Italy and Germany.

“This collaborative project is a great example of how advocacy and academia can work together with private and public sectors to gain further insight into Rett syndrome, allowing us to share this insight with the communities affected by this disease,” said Stefan Weber, CEO of Newron Pharmaceuticals.



### 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 characterised 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.rettssyndrome.org](http://www.rettssyndrome.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 NW-3509 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](http://www.newron.com)

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