



## **Newron Initiates Outreach on First Global Rett Syndrome Burden of Illness Survey in Collaboration with the Rett Community**

***Sponsoring the largest, most comprehensive qualitative study ever to examine the burden of Rett syndrome on individuals and their caregivers***

***Survey outreach to begin in the United States***

**Milan, Italy and Morristown, NJ, USA, October 29, 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, as part of its commitment to finding a treatment for the symptoms of Rett syndrome, is partnering with the global Rett community to sponsor the first-ever International Rett Syndrome Burden of Illness Survey.

Rett syndrome is a severe neuro-developmental orphan disease with no approved treatment options and overwhelmingly affects girls starting at a very young age. The survey outreach, to launch November 1<sup>st</sup> in the United States and in early 2020 in Europe under the banner "[Voices of Rett](#)," aims to deliver data and analytics to quantify the physical, emotional and financial challenges of Rett syndrome on patients, their families, caregivers and the entire Rett patient community. The results will help identify and guide improved intervention programs and services designed to complement the Rett care pathway.

"The symptoms of Rett syndrome are often overwhelming for patients, families and caregivers," said Melissa Kennedy, Executive Director of [Rettsyndrome.org](#). "Individuals with Rett syndrome have very different physical challenges and needs from other illnesses. Today, there are few resources available for Rett patients and their families. Each family is left to carve their own way to find the resources they need to care for their loved one. That is why this survey is vitally needed. It will help provide a sound understanding of the impact of Rett syndrome on patients and their caregivers. It's a valuable opportunity for the global Rett community to make their voices heard by those influencing the development, use and funding of treatment."

"This comprehensive caregiver and healthcare provider survey will be the most accurate and meaningful way of assessing the overall burden of this illness," said Dennis Dionne, Newron Pharmaceutical's Vice President of Commercial Affairs. "It will provide crucial, objective information to policy-makers, payers, and other healthcare system decision-makers so that they can better support the resources, services and therapies needed for Rett syndrome patients and caregivers."

This international Burden of Illness Survey is being conducted by an independent, international Clinical Research Organization in collaboration with Rett syndrome advocacy groups worldwide, along with an advisory panel of experts comprised of physicians, caregivers, advocacy representatives and academic researchers. A scientific poster on the design of this groundbreaking study has been presented at the [26<sup>th</sup> Annual Conference of the International Society for Quality of Life Research \(ISOQOL\)](#) in San Diego, CA on October 22, 2019. The final results of this international survey will be published in academic journals to help increase awareness of the burden of illness for Rett syndrome.



Those eligible to participate in the survey include current primary caregivers (including parents, grandparents, siblings, and paid caregivers) of at least one girl/woman with Rett syndrome who is being cared for at least 10 hours per week; and healthcare providers (including physicians, nurses and allied healthcare professionals) who have treated at least five individuals with Rett syndrome in the past five years as part of their practice. Caregivers and healthcare providers are encouraged to visit <https://voicesofrett.org/> for more specifics on eligibility and to participate in the survey. The active link to the survey will be available to U.S. participants beginning on November 1, 2019.

Newron Pharmaceuticals is developing a treatment to improve the daytime breathing complications, or apneas, of Rett syndrome patients. Apneas are a particularly debilitating core symptom of Rett syndrome, present in approximately 70 percent of patients, and contribute significantly to other comorbidities, as well as to a reduced quality of life. Newron's pivotal STARS clinical study (Sarizotan Treatment of Apneas in Rett Syndrome) in patients with Rett syndrome has completed enrollment and Newron expects to report results from the STARS study during the 4<sup>th</sup> quarter of 2019.

#### **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 <https://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's 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, XETRA: NP5) 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, Canada, Brazil, the United Arab Emirates and Japan, 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®/safinamide 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](http://www.newron.com)

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