



Newron Expands Global Rett Syndrome Burden of Illness Survey Outreach to Europe and Australia

Today's expansion, in close collaboration with Rett advocacy community, represents the largest, most comprehensive qualitative study ever to examine the burden of Rett syndrome on individuals and their caregivers

Milan, Italy and Morristown, NJ, USA, Feb. 17, 2020 -- [Newron Pharmaceuticals S.p.A.](#) ("Newron") (SIX: NWRN, XETRA: NP5), 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 patient advocacy community to sponsor the first-ever International Rett syndrome Burden of Illness (BOI) survey, to take place in Europe and Australia.

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 was initially launched in the United States in November 2019 and has achieved strong participation among Rett patient caregivers and medical professionals.

The Burden of Illness survey outreach will launch outside the U.S. today in the UK, Germany, Italy and Australia under the banner "[Voices of Rett](#)," and 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 to complement the Rett care pathway. For the first time ever, an estimate will be made on the economic impact of a disease on a country, its specific regions, its communities, and even individuals. As new advances in Rett are developed, this knowledge and substantiation will help policy makers and the community support the direct and indirect expenditures needed for the treatment and management of Rett patients.

"We are very happy to be able to expand the outreach for our Burden of Illness survey on an international level," said Stefan Weber, CEO of Newron Pharmaceuticals. "We are grateful that this study is strongly supported by so many Rett patient advocacy groups."

"Very little information concerning the true cost of Rett syndrome is available," said Rachael Stevenson, Executive Director of [Reverse Rett \(UK\)](#). "As new advances in treatments for Rett are developed, the community urgently needs this information to support the case for funding."

"The Rett syndrome BOI survey provides a great opportunity for families to help focus the spotlight on the devastating impact of the disorder on the person they love, but also on the impact it has on the whole family," added Becky Jenner, CEO of [Rett UK](#). "With no dedicated treatment for any aspect of Rett syndrome, the study will help the whole community understand just why a treatment, even if it helps with just one symptom, is so very important."

"The survey is key so we can capture and publish individual caregiving situations for the very first time," said Gabriele Keßler, First Chairman of the Federal Association, [Rett Deutschland e.V.](#) "Care for these patients often depends heavily on whether the parents have the wherewithal to advocate for the rights of the child with Rett syndrome."



“Even some of our treating physicians have a different view than the families on how Rett syndrome can impact our children’s lives and their daily needs,” explained Orietta Mariotti, Advisor, [ProRETT Ricerca](#) in Italy. “The BOI survey will help bridge this gap to support the needs of Rett children and their families around the globe.”

“Australian families can contribute significantly to this large international study to better understand the physical, emotional and financial impacts of Rett syndrome,” said Dr. Jenny Downs, Head of Child Disability Health and Wellbeing for the [Telethon Kids Institute in Australia](#). “This will provide critical information to guide how researchers, professionals and agencies, as well as industries work to find new supports and therapies for our children.”

“There is a need for a strong family voice to describe the impacts of Rett syndrome symptoms on our families,” said Claude Buda, President of [Rett Syndrome Association of Australia](#). “This study will build community, professional, NDIS and government knowledge of the most needed supports necessary to live daily with Rett syndrome in Australia.”

The International BOI 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 [26th 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 three individuals with Rett syndrome in the past five years as part of their practice. Caregivers and healthcare providers seeking to participate in the Survey are encouraged to visit <https://voicesofrett.org/> and look for the flag for your specific country at the top, right of the home page.

Newron Pharmaceuticals is developing a treatment to improve the daytime breathing complications, or apneas and their associated impact on overall symptoms 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 co-morbidities, 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 first half of 2020.

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, please visit the following websites:

UK: <https://www.reverserett.org.uk/> or <https://www.rettuk.org/>



Germany: <https://www.rett.de/de/>

Italy: <https://prorett.org/>

Australia: <https://rett.telethonkids.org.au/> or <https://www.rettaustralia.com/>

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 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, Colombia, 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

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