



## **Newron Supports Global Rare Disease Day® 2018 and Rett Syndrome Studies**

### ***Sponsoring Comprehensive Study to Examine the Burden of Rett Syndrome on Patients and Caregivers; Supporting Standardized Methodology for Health Economic Assessment of Orphan Drugs***

**Milan, Italy and Morristown, NJ, USA, February 13, 2018** - [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 its support of this year's Rare Disease Day®. Observed on February 28, Rare Disease Day helps raise awareness of rare diseases, and in turn improve access to treatments.

As part of its commitment to the rare disease patient community, Newron is partnering with the global Rett community to work on the first Burden of Disease (BOD) study. The study aims to deliver data and analytics to quantify the physical, emotional and financial challenges of Rett syndrome. The learnings accrued will identify and guide improved intervention programs and services designed to complement the Rett care pathway.

Newron, in collaboration with industry and university leaders, is advocating the development of a standardized evaluation method for the health economic assessment of orphan drugs. Most recently, for example, the Company hosted [a roundtable discussion](#) on the opportunity to develop quality clinical, economic and real-world evidence (RWE: data collected in routine clinical practice on how patients are being managed for a disease) on orphan drugs to inform policy decisions. The lack of an accepted method to assess the value of rare disease treatments can be an unsurmountable barrier to their development and/or access once approved.

"There are considerable unmet needs as only 5% of rare diseases have an approved treatment," stated Dennis Dionne, Vice President Commercial Affairs, Newron. "For orphan drugs being developed, there are significant challenges in assessing the clinical and economic value. Understanding the real value of such drugs in terms of clinical benefit and improving quality-of-life (QoL) for patients and their families is crucial for payers to be able to assess their overall benefit against the cost of treatment. We are proud to engage in a meaningful way in Rare Disease Day, and to help advocate for improved methodologies and access."

#### **For more information**

See interviews with leading Rett-experts: <http://www.newron.com/ENG/Default.aspx?PAG=215>

#### **STARS Study**

Newron has also initiated the Sarizotan Treatment of Apneas in Rett Syndrome (STARS) study, a potentially pivotal 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 Rare Disease Day®**

Rare Disease Day takes place on the last day of February each year, thus on February 28, 2018. The main objective of Rare Disease Day is to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives. The campaign targets primarily the general public and also seeks to raise awareness amongst policy makers, public authorities, industry representatives, researchers, health professionals and anyone who has a genuine interest in rare diseases. The campaign started as a European event and has progressively become a world phenomenon, with the USA joining in 2009 and participants in over 80 countries throughout the world in 2016.



For more information, please visit: [www.rarediseaseday.org](http://www.rarediseaseday.org).

#### **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: [www.newron.com](http://www.newron.com)

#### **For more information**

##### **Newron**

Stefan Weber – CEO  
+39 02 6103 46 26  
[pr@newron.com](mailto:pr@newron.com)

##### **UK/Europe**

Julia Phillips / Natalie Garland-Collins, FTI Consulting  
+44 20 3727 1000  
[SCnewron@fticonsulting.com](mailto:SCnewron@fticonsulting.com)

##### **Switzerland**

Martin Meier-Pfister, IRF Communications  
+41 43 244 81 40  
[martin.meier-pfister@irfcom.ch](mailto:martin.meier-pfister@irfcom.ch)

##### **Germany/Europe**

Anne Hennecke, MC Services  
+49 211 52925222  
[anne.hennecke@mc-services.eu](mailto:anne.hennecke@mc-services.eu)

##### **USA**

Beth Kurth, LaVoieHealthScience  
+1 617 374 8800, Ext. 106  
[bkurth@lavoiehealthscience.com](mailto:bkurth@lavoiehealthscience.com)

#### **Important Notices**

This document contains forward-looking statements, including (without limitation) about (1) Newron's ability to develop and expand its business, successfully complete development of its current product candidates and current and future collaborations for the development and commercialisation of its product candidates and reduce costs (including staff costs), (2) the market for drugs to treat CNS diseases and pain conditions, (3) Newron's anticipated future revenues, capital expenditures and financial resources, and (4) assumptions underlying any such statements. In some cases, these statements and assumptions can be identified by the fact that they use words such as "will," "anticipate," "estimate,"



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