



## **Newron Pharmaceuticals Supports Rare Disease Day® and Joins Global Movement to Raise Awareness for Rare Diseases**

**Milan, Italy, February 29, 2016** - Newron Pharmaceuticals S.p.A. ("Newron"), a biopharmaceutical company focused on the development of novel therapies for patients with diseases of the central nervous system (CNS) and pain, today announced it has joined forces with 300 million people living with rare diseases and health care advocates around the world for Rare Disease Day®, an annual global initiative dedicated to elevating public understanding of rare diseases and calling attention to the special challenges of people affected by these.

"We are proud to support NORD (The National Organization for Rare Disorders in the USA) and EURORDIS (the European Organization for Rare Diseases) to raise awareness for rare diseases and their devastating impact on patients' lives," said Stefan Weber, CEO of Newron, "We are particularly honored to support groups such as Rettsyndrome.org and Curerett.org, who are dedicated to improving the lives of girls living with Rett syndrome, which is one of more than 6,000 rare diseases."

### **About Rare Disease Day®**

Rare Disease Day was launched by EURORDIS and its Council of National Alliances in 2008. Held on the last day of February each year, a rare day, it seeks to raise awareness of the impact that rare diseases have on the lives of patients and those who care for them. What began as a European event quickly became international in scope, with participants from more countries joining each year. See the [Rare Disease Day 2016 video](#).

Since Rare Disease Day began, thousands of events have been held throughout the world, reaching hundreds of thousands of people. The political momentum resulting from the Day has also served advocacy purposes, contributing to the advancement of EU policies on rare diseases and the creation of national plans for rare diseases in a number of EU Member States and now in other countries.

In the United States, the National Organization for Rare Disorders (NORD) promotes and celebrates Rare Disease Day. For more information about Rare Disease Day in the U.S., please visit <http://www.rarediseaseday.us/>.

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

### **About Rare Diseases**

The European Union considers a disease as rare when it affects fewer than 1 in 2,000 citizens. According to EURORDIS, over 6000 different rare diseases have been identified to date, affecting over 60 million people in Europe and the USA alone. Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offering inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.

Newron is pursuing clinical development with sarizotan in Rett syndrome.

### **About Rett Syndrome**

Rett syndrome is a severe neurodevelopmental disorder primarily affecting females, with an estimated prevalence ranging from one in 10,000 to one in 20,000 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. Marketing authorization in the EU for Xadago® (safinamide) for the treatment of Parkinson's disease was granted by the EU Commission in February 2015, followed by Swissmedic's marketing authorization for Switzerland in November 2015. The drug has been launched by Newron's partner Zambon in the first key EU countries Germany, Spain and Italy, as well as in Switzerland. The New Drug Application (NDA) has been accepted for review by the FDA, PDUFA date March 29, 2016. Zambon has the rights to develop and commercialize safinamide globally, excluding Japan and other key Asian territories, where Meiji Seika has the rights to develop and commercialize the compound. Newron's additional projects are based on highly promising treatments for rare disease patients and are at various stages of clinical development. They include Sarizotan for patients with Rett Syndrome, for which Newron received Orphan Drug Designation in both the US and the EU, ralfinamide for patients with specific rare pain indications, and NW-3509 as potentially the first add-on therapy for the treatment of patients with positive symptoms of schizophrenia.

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#### **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", "expect", "project", "intend", "plan", "believe", "target", and other words and terms of similar meaning. All statements, other



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Newron does not undertake any obligation to publicly update or revise forward-looking statements except as may be required by applicable regulations of the SIX Swiss Exchange, where the shares of Newron are listed.

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