Newron Reports Top-Line Results from its STARS Study Evaluating Sarizotan in Patients with Rett Syndrome

*Pivotal trial did not meet primary or secondary efficacy variables*

*Newron will prioritize its Phase III development program of Evenamide in schizophrenia and evaluate additional pipeline candidates*

Milan, Italy and Morristown, NJ, USA, May 4, 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, announced today that top-line results from its STARS clinical study evaluating sarizotan in patients with Rett syndrome did not demonstrate evidence of efficacy on the primary or secondary efficacy variables. Consequentially, Newron has decided to terminate this development program.

The STARS (Sarizotan for the Treatment of Apneas in Rett Syndrome) clinical study qualified and enrolled 129 Rett syndrome patients in 14 centres throughout the US, Europe, Asia and Australia for the six-month clinical trial. Patients received treatment with daily doses of 10 mg and 20 mg of sarizotan, or placebo. The primary endpoint of the STARS study was a percentage reduction in episodes of apnea during waking time compared with placebo. Newron plans to work with the Rett research community and families to share learnings from the STARS clinical study as well as from the Rett Syndrome International Burden of Illness Survey, to further advance scientific and medical understanding of this disease.

Ravi Anand, M.D., Chief Medical Officer of Newron, commented: “We are very disappointed that the top-line results in the STARS study did not meet the study endpoints. The results of this well designed and executed study, based on highly promising data from a genetic model of Rett syndrome in mice, indicate the difficulties inherent in translating effects in animal models to human clinical studies. We are currently awaiting results of additional explanatory analyses and will continue to analyse the full data set from the study to understand more about the results.”

Stefan Weber, Newron’s Chief Executive Officer, said: “We sincerely want to thank the patients, caregivers and their families who participated in this landmark study. This was an extremely challenging study and the enthusiasm of the patients and caregivers indicates their need to find a treatment for this serious, underserved disease. Each participant has the gratitude of the study investigators, Newron’s employees, advisors and vendors who have worked diligently on this study over the last five years.”

“Newron continues to develop its pipeline and evaluate additional candidates, consistent with its long-term strategy of developing novel therapies for patients with diseases of the central and peripheral nervous system. In the near term, we look forward to progressing our Phase III clinical program evaluating Evenamide in schizophrenia.”
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.

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 currently holds the commercialization rights in the USA and has entered into a definitive agreement to sell its Xadago rights, along with other CNS assets, to Supernus Pharmaceuticals. Meiji Seika has the rights to develop and commercialize the compound in Japan and other key Asian territories. 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, please contact

Newron
Stefan Weber – CEO
+39 02 6103 46 26
pr@newron.com

UK/Europe
Simon Conway / Natalie Garland-Collins, FTI Consulting
+44 20 3727 1000
SCnewron@fticonsulting.com

Switzerland
Martin Meier-Pfister, IRF
+41 43 244 81 40
meier-pfister@irf-reputation.ch

Germany/Europe
Anne Hennecke, MC Services
+49 211 52925222
anne.hennecke@mc-services.eu

USA
Paul Sagan, LaVoieHealthScience
+1 617 374 8800, Ext. 112
psagan@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,” “expect,” “project,” “intend,” “plan,” “believe,” “target,” and other words and
terms of similar meaning. All statements, other than historical facts, contained herein regarding Newron’s strategy,
goals, plans, future financial position, projected revenues and costs and prospects are forward-looking statements.
By their very nature, such statements and assumptions involve inherent risks and uncertainties, both general and
specific, and risks exist that predictions, forecasts, projections and other outcomes described, assumed or implied
therein will not be achieved. Future events and actual results could differ materially from those set out in,
contemplated by or underlying the forward-looking statements due to a number of important factors. These factors
include (without limitation) (1) uncertainties in the discovery, development or marketing of products, including
without limitation negative results of clinical trials or research projects or unexpected side effects, (2) delay or
inability in obtaining regulatory approvals or bringing products to market, (3) future market acceptance of products,
(4) loss of or inability to obtain adequate protection for intellectual property rights, (5) inability to raise additional
funds, (6) success of existing and entry into future collaborations and licensing agreements, (7) litigation, (8) loss
of key executive or other employees, (9) adverse publicity and news coverage, and (10) competition, regulatory,
legislative and judicial developments or changes in market and/or overall economic conditions. Newron may not
actually achieve the plans, intentions or expectations disclosed in forward-looking statements, and assumptions
underlying any such statements may prove wrong. Investors should therefore not place undue reliance on them.
There can be no assurance that actual results of Newron’s research programmes, development activities,
commercialisation plans, collaborations and operations will not differ materially from the expectations set out in
such forward-looking statements or underlying assumptions. 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. This announcement is not an offer for sale of securities in the
United States, Canada, Australia or Japan or any other jurisdiction where such an offer or solicitation would
otherwise be unlawful. The securities referred to herein may not be sold in the United States absent registration or
an exemption from registration under the U.S. Securities Act of 1933, as amended. Newron does not intend to
register any of its securities in the United States or to conduct a public offering of its securities in the United States.
This document does not contain or constitute an offer or invitation to purchase or subscribe for any securities of
Newron and no part of this document shall form the basis of or be relied upon in connection with any contract or
commitment whatsoever.