Neuren (NEU) - ASX Announcement **31 October 2019**

Phase 3 trial of trofinetide in Rett syndrome commences

Melbourne, Australia, 31 October 2019: Neuren Pharmaceuticals (ASX: NEU) today announced that its North American licensee ACADIA Pharmaceuticals has commenced the Phase 3 trial of trofinetide in Rett syndrome in the United States. The US Food and Drug Administration (FDA) has granted Orphan Drug designation and Fast Track designation for the Rett syndrome program. ACADIA's announcement of initiation of the trial, including more details about the trial and about Rett syndrome is attached.

The Phase 3 trial and all associated development costs are fully funded by ACADIA and Neuren has free and full access to all data for use to commercialise outside North America.

Neuren Executive Chairman Richard Treagus commented: "The commencement of Phase 3 is a very important milestone for all stakeholders as we work towards achieving an approved treatment for patients and families living with the debilitating effects of Rett syndrome. We are very pleased with the progress ACADIA has made since we announced our partnership last year. With our lead drug trofinetide now in Phase 3 and our second drug NNZ-2591 moving into clinical trials for three separate Orphan Drug designated childhood disorders in 2020, Neuren is in a fundamentally strong position with a diverse and valuable pipeline."

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is developing new therapies for neurodevelopmental disorders with high unmet need, utilizing synthetic analogs of neurotrophic peptides that occur naturally in the brain. Neuren's lead drug candidate trofinetide is in a Phase 3 clinical trial for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. The programs in Rett syndrome and Fragile X syndrome have each received Fast Track designation by the US Food and Drug Administration and Orphan Drug designation in both the United States and the European Union. Neuren has granted an exclusive license to ACADIA Pharmaceuticals Inc. for the development and commercialization of trofinetide in North America, whilst retaining all rights outside North America. Neuren is advancing the development of its second drug candidate NNZ-2591 for Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome, each of which has Orphan Drug designation in the United States.

Forward-looking Statements
This announcement contains forward-looking statements that are subject to risks and uncertainties. Such statements involve known and unknown risks and important factors that may cause the actual results, performance or achievements of Neuren to be materially different from the statements in this announcement.

Contact:

Jon Pilcher, CFO & Company Secretary: jpilcher@neurenpharma.com; +61 438 422 271

ACADIA Pharmaceuticals Initiates Phase 3 Pivotal Study of Trofinetide in Rett Syndrome, a Rare Neurodevelopmental Congenital CNS Disorder

October 30, 2019 04:15 PM Eastern Daylight Time

SAN DIEGO--(BUSINESS WIRE)--ACADIA Pharmaceuticals Inc. (Nasdaq: ACAD), a biopharmaceutical company focused on the development and commercialization of innovative medicines to address unmet medical needs in central nervous system (CNS) disorders, today announced that it has initiated the Phase 3 LAVENDER placebo-controlled study to evaluate the efficacy and safety of trofinetide for girls and young women with Rett syndrome. Rett syndrome is a serious and rare neurodevelopmental congenital CNS disorder with symptoms that typically present between six to 18 months of age, and lead to problems with cognitive, sensory, motor, and autonomic function.

“There is no approved treatment for Rett syndrome, which is a rare neurological disease that impacts nearly every aspect of a child’s life, resulting in loss of speech, difficulty breathing, lack of motor control, loss of muscle tone and mobility, seizures, and more,” said Jeffrey L. Neul, M.D., Ph.D., Annette Schaffer Eskind Chair and Director, Vanderbilt Kennedy Center; Professor of Pediatrics, Division of Neurology, Pharmacology, and Special Education, Vanderbilt Kennedy Center, Vanderbilt University Medical Center and LAVENDER study investigator. “I look forward to the outcomes of this clinical program evaluating trofinetide as a potentially new treatment for Rett syndrome.”

The LAVENDER Phase 3 study is a 12-week, double-blind, randomized, placebo-controlled study evaluating the efficacy and safety of trofinetide in approximately 180 girls and young women 5 to 20 years of age with Rett syndrome. Half of study participants will receive trofinetide and half will receive placebo. Co-primary efficacy endpoints of the study will measure symptom improvement using the Rett Syndrome Behavior Questionnaire (RSBQ), a caregiver assessment, and the Clinical Global Impression Scale-Improvement (CGI-I), a clinician assessment.

“For patients living with this debilitating disease, and the families whose dedication to their care inspires us, the LAVENDER study is an important next step in what we hope will result in the first FDA-approved treatment for Rett syndrome,” said Serge Stankovic, M.D., M.S.P.H., ACADIA’s President. “We are grateful to study participants and their families, investigators, Rettsyndrome.org, and Neuren Pharmaceuticals who have played instrumental roles in advancing trofinetide to this stage of clinical development and look forward to building upon this work to further evaluate trofinetide in the Phase 3 LAVENDER study.”

The LAVENDER study will be followed by LILAC, a nine-month extension study in which all participants, including those on placebo in the LAVENDER study, will be eligible to receive trofinetide. All LILAC participants will be followed to evaluate long-term tolerability, safety, and effectiveness of trofinetide. A second extension study, LILAC-2, will follow in which eligible patients who complete the LILAC trial will continue to receive trofinetide.

“The start of the trofinetide study has been highly anticipated by the Rett community, which currently has no approved treatment for Rett syndrome,” said Melissa Kennedy, Executive Director of RettSyndrome.org (RSO). “We are hopeful for what this study means for patients and their families as it potentially brings us closer to improving the lives of many living with Rett syndrome.”

In 2018, ACADIA entered into an exclusive North American license agreement with Neuren for the development and commercialization of trofinetide for Rett syndrome and other indications. In an end-of-Phase 2 meeting, the FDA confirmed that positive results from a pivotal Phase 3 study for trofinetide in Rett syndrome and the extension study could be the basis of a New Drug Application (NDA) submission.

More information about the LAVENDER study is available at www.rettsyndromestudies.com.

About Trofinetide

Trofinetide is an investigational drug. It is a novel synthetic analog of the amino-terminal tripeptide of IGF-1 designed to treat the core symptoms of Rett syndrome by potentially reducing neuroinflammation and supporting synaptic function. In the central nervous system, IGF-1 is produced by both of the major types of brain cells – neurons and glia. IGF-1 in the brain is critical for both normal development and for response to injury and disease. Trofinetide has been granted Fast Track Status and Orphan Drug Designation in the U.S. and Orphan Drug Designation in Europe for both Rett syndrome and Fragile X syndrome.

About Rett Syndrome

Rett syndrome is a debilitating neurological disorder that occurs primarily in females following apparently normal development for the first six months of life. Rett syndrome has been most often misdiagnosed as autism, cerebral palsy, or non-specific developmental delay. Rett syndrome is caused by mutations on the X chromosome on a gene called *MECP2*. There are more than 200 different mutations found on the *MECP2* gene that interfere with its ability to generate a normal gene product. Rett syndrome occurs worldwide in approximately one of every 10,000 to 15,000 female births causing problems in brain function that are responsible for cognitive, sensory, emotional, motor and autonomic function. Typically, between six to 18 months of age, patients experience a period of rapid decline with loss of purposeful hand use and spoken communication and inability to independently conduct activities of daily living. Symptoms also include seizures, disorganized breathing patterns, an abnormal side-to-side curvature of the spine (scoliosis), and sleep disturbances. Currently, there are no FDA-approved medicines for the treatment of Rett syndrome.

About ACADIA Pharmaceuticals

ACADIA is a biopharmaceutical company focused on the development and commercialization of innovative medicines to address unmet medical needs in central nervous system disorders. ACADIA has developed and commercialized a treatment for hallucinations and delusions associated with Parkinson's disease psychosis. In addition, ACADIA has ongoing clinical development efforts in additional areas with significant unmet need, including dementia-related psychosis, schizophrenia-negative symptoms, major depressive disorder, and Rett syndrome. This press release and further information about ACADIA can be found at www.acadia-pharm.com.

Forward-Looking Statements

Statements in this press release that are not strictly historical in nature are forward-looking statements. These statements include but are not limited to statements regarding the timing of the commencement of the Phase 3 clinical trial evaluating trofinetide; the likelihood of success of such clinical trial; the prospects for FDA approval of trofinetide for Rett syndrome and other indications; and the success of any efforts to commercialize trofinetide in North America. These statements are only predictions based on current information and expectations and involve a number of risks and uncertainties. Actual events or results may differ materially from those projected in any of such statements due to various factors, including the risks and uncertainties inherent in drug discovery, development, approval and commercialization. For a discussion of these and other factors, please refer to ACADIA’s annual report on Form 10-K for the year ended December 31, 2018 as well as ACADIA’s subsequent filings with the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. This caution is made under the safe harbor

provisions of the Private Securities Litigation Reform Act of 1995. All forward-looking statements are qualified in their entirety by this cautionary statement and ACADIA undertakes no obligation to revise or update this press release to reflect events or circumstances after the date hereof, except as required by law.

Contacts

Investor Contact:
ACADIA Pharmaceuticals Inc.
Mark Johnson, CFA
(858) 261-2771
ir@acadia-pharm.com

Media Contact:
ACADIA Pharmaceuticals Inc.
Maurissa Messier
(858) 768-6068
media@acadia-pharm.com