ACADIA Pharmaceuticals and Neuren Pharmaceuticals Announce Rare Pediatric Disease Designation for Trofinetide for the Treatment of Rett Syndrome

With No Approved Treatments in Rett Syndrome, FDA Decision Highlights Significant Unmet Need

SAN DIEGO, CA & MELBOURNE, Australia - March 3, 2020 - ACADIA Pharmaceuticals Inc. (Nasdaq: ACAD) and Neuren Pharmaceuticals Limited (ASX: NEU) announced today that the U.S. Food and Drug Administration (FDA) granted Rare Pediatric Disease (RPD) designation to trofinetide for the treatment of Rett syndrome, a serious and rare neurological disorder. Upon FDA approval of a product with RPD designation, the sponsor may be eligible to receive a Priority Review Voucher, which can be used to obtain FDA review of a New Drug Application for another product in an expedited period of six months.

"We are pleased that the FDA has recognized the unmet need currently experienced by Rett patients and their families and our goal is to bring a treatment option forward as soon as possible," said Serge Stankovic, M.D., M.S.P.H., ACADIA's President. "This is an encouraging step forward as we continue to enroll patients in our Phase 3 LAVENDER study with results expected in 2021."

RPD designation is granted by the FDA in the case of serious or life-threatening diseases affecting fewer than 200,000 people in the U.S. and primarily in individuals 18 years of age and younger. Trofinetide was previously granted Fast Track Status and Orphan Drug Designation for Rett syndrome in the U.S. and Orphan Drug Designation for Rett syndrome in Europe.

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 and in the United States impacts 6,000 to 9,000 patients. Rett syndrome causes problems in brain function that are responsible for cognitive, sensory, emotional, motor and autonomic function. Typically, with symptoms presenting 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 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 Rare Pediatric Disease Designation for the treatment of Rett Syndrome in the U.S. and Orphan Drug Designation in the U.S. and Europe for both Rett syndrome and Fragile X syndrome.

The Phase 3 trofinetide clinical program includes LAVENDER, a 12-week, double-blind, placebo-controlled study, and LILAC, an open-label, long-term extension study. The Phase 3 clinical program is progressing as planned with 11 study sites recruiting and more sites expected in the future.

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 the first and only medicine approved for the treatment of hallucinations and delusions associated with Parkinson's disease psychosis. ACADIA also has ongoing clinical development efforts in additional areas with significant unmet need, including dementia-related psychosis, the negative symptoms of schizophrenia, major depressive disorder, and Rett syndrome. This press release and further information about ACADIA can be found at www.acadia-pharm.com.

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. Under the terms of the license agreement between ACADIA and Neuren, Neuren is eligible to receive one third of the market value of any Rare Pediatric Disease Priority Review Voucher, if awarded by the U.S. FDA upon approval of a New Drug Application for trofinetide.

About Neuren Pharmaceuticals

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. Neuren has completed Phase 2 development of trofinetide for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome.

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 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,

2019 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.

This ASX-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.

ACADIA Pharmaceuticals Inc.

Media Contact:
Stephanie Fagan
(858) 212-0534
media@acadia-pharm.com
or
Investor Contact:
Mark Johnson, CFA
(858) 261-2771
ir@acadia-pharm.com
or
Neuren Pharmaceuticals Limited
Contact:
Jon Pilcher
Chief Financial Officer & Company Secretary
+61 438 422 271
jpilcher@neurenpharma.com