4 February 2019

The Company Announcements Office
ASX Limited

Plans for Phase 3 trial of trofinetide in Rett syndrome

The attached statement has been released today by ACADIA Pharmaceuticals regarding plans for the Phase 3 trial of trofinetide in Rett syndrome, starting in the second half of 2019.

Jon Pilcher
CFO & Company Secretary
Neuren Pharmaceuticals

Dear Rett Community,

We would like to share our plans for the Phase 3 study of trofinetide for Rett syndrome starting in the second half of 2019 which have now been confirmed.

ACADIA plans to conduct a three-month Phase 3, double-blind, randomized, placebo-controlled study to evaluate efficacy and safety of trofinetide and placebo in approximately 180 females ages 5 to 20 years with Rett syndrome. Half of the study participants will receive trofinetide and half will receive placebo. The study will use the Rett Syndrome Behaviour Questionnaire (RSBQ), a caregiver assessment and the Clinical Global Impression Scale-Improvement (CGI-I), a clinician assessment of the improvement of Rett syndrome as co-primary efficacy endpoints.

The Phase 3 study will be followed by an open label extension study in which all participants, including those on placebo in the Phase 3 study, will be eligible to receive trofinetide. In the open label extension study, all participants will be followed to evaluate long term tolerability and safety of trofinetide.

For participants aged 18 to 20 years, legal guardianship must be in place for a parent/caregiver to give consent for participation in the trial. Females who participated in previous trofinetide studies may be eligible to participate in the Phase 3 study if all of the inclusion criteria and none of the exclusion criteria are met.

ACADIA appreciates the feedback provided by Neuren Pharmaceuticals, investigators from the Phase 2 studies completed by Neuren, Rettsyndrome.org (RSO), and the U.S. Food and Drug Administration (FDA) to further optimize the Phase 3 trial design.

As you know, today there are no approved medicines for the treatment of Rett syndrome. ACADIA is committed to thoroughly and expeditiously evaluating trofinetide for Rett syndrome. With positive results, we plan to submit to the FDA a New Drug Application (NDA) in 2021.

We will continue to provide updates when possible about the Phase 3 study of trofinetide for Rett syndrome. Please also visit us at http://www.acadia-pharm.com/pipeline/rett-syndrome/ for more information about Rett syndrome and our trofinetide clinical trial program.

The ACADIA Rett Team