May 1, 2019

Dear Rett Community,

We are on track to initiate the LAVENDER 12-week Phase 3 placebo-controlled study in the fourth quarter of 2019. This study will evaluate the efficacy and safety of trofinetide and placebo in approximately 180 females ages 5 to 20 years with Rett syndrome.

It will be randomized with half of the study participants receiving trofinetide and half receiving placebo, and 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 LAVENDER Phase 3 study will be followed by LILAC, a 40-week open-label extension study. All participants completing the 12-week LAVENDER study may be eligible to enroll in the LILAC study, in which all study participants will receive trofinetide and followed to evaluate long term tolerability and safety of the drug.

Please continue to visit us at http://www.acadia-pharm.com/pipeline/rett-syndrome/ for information about Rett syndrome and our trofinetide clinical program.

For any questions about the Phase 3 trofinetide study, please contact our team at medicalinformation@acadia-pharm.com.

The ACADIA Rett Team