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The Company Announcements Office
ASX Limited

Update on trofinetide Phase 3 trial in Rett syndrome

The attached update has been released today by ACADIA Pharmaceuticals regarding the Phase 3 LAVENDER and LILAC studies progressing in the United States.

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ASX Listing Rules information
This announcement was authorized to be given to the ASX by the board of directors of Neuren Pharmaceuticals Limited, Suite 201, 697 Burke Road, Camberwell, VIC 3124

Dear Rett Community,

In recognition of Rare Disease Day this month, we are excited to provide an update on the trofinetide Phase 3 LAVENDER study and the LILAC extension study. As we have shared before, LAVENDER is a 12-week study that will evaluate the efficacy and safety of trofinetide and placebo in approximately 180 girls and young women aged 5 to 20 years with Rett syndrome. All girls and young women completing the LAVENDER trial are eligible to enroll in the LILAC study, a 40-week extension study in which all participants receive trofinetide and are followed to evaluate long term tolerability, safety, and effectiveness of the drug.

The Phase 3 trofinetide program is progressing as planned. The pivotal, placebo-controlled LAVENDER study and the open-label extension LILAC study are both clinically active and enrollment is underway.

Eleven study sites have started recruiting for the LAVENDER study and more sites are expected to open in the coming weeks. These open study sites are located in the following cities:

- Aurora, CO
- Birmingham, AL
- Boston, MA
- Chicago, IL
- Cincinnati, OH
- Houston, TX
- La Jolla, CA
- Nashville, TN
- Phoenix, AZ
- St. Louis, MO
- St. Paul, MN

We anticipate that the remaining sites in Cleveland, OH, Greenwood, SC, New York City, NY, and Philadelphia, PA will be recruiting in the coming weeks.

If you have visited the LAVENDER study web site (www.rettsyndromestudies.com) and registered participation of interest, your information has been appropriately recorded and passed on to the closest clinical trial site available. Once the trial site is ready to begin the study, a site coordinator will be in touch with you.

If you have any questions about trofinetide, please contact us at medicalinformation@acadia-pharm.com.

Thank you for your continued support. Your unwavering dedication to your girls reminds us each day why we are pursuing the development of an effective treatment for Rett syndrome.

All our best,
The ACADIA Rett Team