February 28, 2023

Dear Rett Community,

This week we shared the topline results of the LILAC-1 study on our Investors Full Year 2022 earnings call. The LILAC-1 study is the open-label safety extension study that allowed Rett Syndrome patients finishing the Phase 3 LAVENDER study to receive trofinetide, an investigational drug for the treatment of Rett syndrome, for up to 40 weeks.

Acadia is pleased to announce that the study showed continued improvement in Rett symptoms (as measured by the Rett Syndrome Behaviour Questionnaire (RSBQ) and Clinical Global Impression–Improvement (CGI-I)) for the duration of their open-label treatment. Importantly, the improvement in Rett symptoms was also observed for patients who transitioned from placebo to trofinetide for the LILAC-1 study. The overall discontinuation rate was approximately 46%. Discontinuations in the study related to an adverse event of diarrhea were 21% over the 40 weeks. The mean change from the LAVENDER baseline to Week 40 in the LILAC-1 study in RSBQ total score was -7.3 for patients treated with trofinetide in LAVENDER and -7.0 for patients treated with placebo in LAVENDER. Mean CGI-I scores compared to the LILAC-1 baseline at Week 40 were 3.1 and 3.2 for patients treated with trofinetide and placebo in LAVENDER, respectively. The most common adverse events in LILAC-1 were: diarrhea (74.7%) and vomiting (28.6%).

The complete results of the LILAC-1 study will be presented at a future scientific conference later this year.

Finally, the Trofinetide New Drug Application is currently under review by the U.S. Food and Drug Administration, with a March 12th, 2023 action date. We will provide further information at this time.

As always, Acadia would like to thank the clinical investigators and Rett families that participated in this study.

Best regards,
Acadia Rett Team

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