December 6, 2021

Dear Rett Community,

Acadia is pleased to announce positive top-line results from the Phase 3 Lavender™ study of trofinetide, an investigational drug for the treatment of Rett syndrome. You can read Acadia's press release here: https://ir.acadia-pharm.com/news-releases.

The Lavender study evaluated the efficacy and safety of trofinetide in 187 girls and young women aged 5-20 years with Rett syndrome. The 12-week placebo-controlled study demonstrated a statistically significant improvement over placebo for both co-primary endpoints. On the Rett Syndrome Behaviour Questionnaire (RSBQ), change from baseline to week 12 was -5.1 vs. -1.7 (*p=0.0175*; effect size = 0.37). The Clinical Global Impression–Improvement (CGI-I) score at week 12 was 3.5 vs. 3.8 (*p=0.0030*; effect size = 0.47). The RSBQ is a caregiver assessment of the core symptoms of Rett syndrome and the CGI-I is a physician assessment of worsening or improving of Rett syndrome.

Trofinetide also met the key secondary endpoint, demonstrating a statistically significant separation over placebo in the Communication and Symbolic Behavior Scales Developmental Profile™ Infant-Toddler Checklist–Social composite score (CSBS-DP-IT–Social) change from baseline to week 12 (-0.1 vs. -1.1; *p=0.0064*; effect size = 0.43). The results from this study will be submitted for presentation at upcoming medical meetings.

Please note, the safety and efficacy of trofinetide have not been reviewed or approved by the U.S. Food and Drug Administration (FDA). As a next step, Acadia is planning for a meeting with the FDA in the first quarter of 2022 and plans to submit a new drug application (NDA) around mid-year 2022.

On behalf of all of us at Acadia, we want to thank the patients, their families, physicians and study site personnel who contributed to making this milestone a reality. As always, we look forward to keeping you updated as we continue our important work with the community.

All our best,

The Acadia Rett Team

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