July 18, 2022

Dear Rett Community,

Acadia is pleased to announce the submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for trofinetide for the treatment of Rett syndrome in adults and pediatric patients 2 years of age and older. The full text of the Acadia press release can be read here: https://ir.acadia.com/news-releases/news-release-details/acadia-pharmaceuticals-submits-new-drug-application-us-fda

The FDA defines an NDA as, *the vehicle through which drug sponsors formally propose that the FDA approve a new pharmaceutical for sale and marketing. Its purpose is to demonstrate that a drug is safe and effective for its intended use in the population studied.*

As noted in the attached release, the NDA submission for trofinetide is supported by results from the Phase 3 Lavender study evaluating the efficacy and safety of trofinetide in 187 girls and young women aged 5-20 years with Rett syndrome, in which a statistically significant improvement over placebo for both co-primary endpoints, the Rett Syndrome Behaviour Questionnaire and the Clinical Global Impression-Improvement scores, was demonstrated.

As always, we remain grateful to the Rett community and the patients and their families who have participated in the trofinetide clinical studies, including our pivotal Phase 3 Lavender study. Your support has made this important milestone possible.

We look forward to working with the FDA as it evaluates the NDA and to keeping you updated on our progress. Additional information about Rett syndrome can be found at Acadia.com/Rett. For questions about trofinetide or clinical trials, please contact us at medicalinformation@acadiapharm.com.

All our best,

The Acadia Rett Team

Acadia Pharmaceuticals Inc.
Tel. 858-558-2871
Fax. 858-558-2872

12830 El Camino Real, Suite #400
San Diego, CA 92130

www.acadia-pharm.com