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Neuren to Conduct First Phase 3 Clinical Trial for Rett Syndrome

(Cincinnati, OH) Rettsyndrome.org is pleased to share that Neuren Pharmaceuticals, today, after meeting with the FDA, will conduct a Phase 3 clinical trial for trofinetide, a compound that shows great promise in the treatment of Rett syndrome, in children and adults. This critical Phase 3 trial will be the final clinical step before FDA approval to become a prescribable drug.

Rettsyndrome.org has worked tirelessly with Neuren Pharmaceuticals, trofinetide's manufacturer, to conduct two separate Phase 2 clinical trials, both of which achieved significant clinical benefit. Trial results for females ages five through 45 largely showed strong evidence of improvement in Rett syndrome symptoms of breathing, hand wringing, and mood. Neuren Pharmaceuticals measured these results utilizing the Rett Syndrome Behavior Questionnaire as well as two other efficacy measures.

For many families, these improvements will revolutionize their child's quality of life. Phase 2 results also provided strong evidence of potential disease modification rather than simply addressing symptoms in isolation. The successful completion of Phase 3 will provide a treatment that is an integral part of Rettsyndrome.org's comprehensive research strategy to correct the biology and reset neurologic function.

Phase 3 will be a double-blind, randomized, placebo-controlled trial and will test one active dose group with a treatment duration of 6 months. The dosing regimen has been designed to achieve consistent drug exposure in subjects regardless of their weight.

Rettsyndrome.org's Chief Science Officer, Steve Kaminsky, PhD, shares "This news is incredibly motivating as Rettsyndrome.org and Neuren now embark on the first phase 3 trial with a drug designed to change Rett biology and improve the lives of those affected."

Neuren's Executive Chairman, Dr. Richard Treagus, comments "We are pleased to have held a very constructive meeting with the FDA Division of Neurology Products. It has provided necessary confirmation on the key issues relating to our proposed Phase 3 trial in Rett syndrome. We are now able to progress the final stages of development with full confidence."

Rettsyndrome.org is relentless in our pursuit of the first-ever prescribable treatment for Rett syndrome.

About Rettsyndrome.org

As the world's leading private funder of Rett syndrome research, Rettsyndrome.org has funded over \$41M in high-quality, peer-reviewed research grants and programs to date. The organization hosts the largest global gathering of Rett researchers and clinicians to establish research direction for the future. Rettsyndrome.org, a 501(c) 3 organization, has earned Charity Navigator's prestigious 3 star rating year after year. To learn more about our work and Rett syndrome, visit www.rettsyndrome.org or call (513) 874-3020.

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