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### **Neuren's Phase 2 trial of trofinetide demonstrates significant clinical benefit in pediatric Rett syndrome**

(Cincinnati, OH) The International Rett Syndrome Foundation, now doing business as Rettsyndrome.org, announces today that Neuren Pharmaceuticals reported that trofinetide had significant clinical benefit in their Phase 2 clinical trial in girls with Rett syndrome aged 5 to 15. Rettsyndrome.org (a sponsor of this trial) is enthusiastic about the results and proud to partner with Neuren to move trofinetide forward toward the Phase 3 clinical trial.

Neuren's trial was a double-blind, randomized, placebo controlled study with 82 subjects. Neuren tested three doses of trofinetide compared with placebo in the subjects. The highest dose of trofinetide achieved statistically significant clinical benefit compared with placebo for each of three syndrome-specific efficacy measures; the Rett Syndrome Behavior Questionnaire, the Clinical Global Impression of Improvement and the Rett Syndrome Domain Specific Concerns. These measures included assessments by both clinicians and caregivers. Clinical improvements above baseline were determined as clinically meaningful by leading Rett syndrome physicians. To read the full study results, please go to <https://www.rettsyndrome.org/document.doc?id=574>

These results provide strong evidence of biological activity of the high dose across multiple symptom areas. This indicates the potential for disease modification rather than simply addressing isolated symptoms. In addition, trofinetide was well tolerated and had a good safety profile in these younger subjects, with no dose-limiting effects observed.

Neuren now intends to discuss with the US Food and Drug Administration (FDA) plans for a pivotal Phase 3 trial commencing in 2018. Neuren will be using the Rett Syndrome Behavior Questionnaire (RSBQ) as a primary efficacy measure, supported by the Clinical Global Impression of Improvement (CGI-I) as a key secondary efficacy measure. In parallel, Neuren will now move to complete the necessary chronic toxicity studies and manufacturing scale-up.

Rettsyndrome.org's Chief Science Officer, Steve Kaminsky, shares, "These pediatric study results are very exciting. The data suggest that trofinetide is having a positive change on a number of challenges of Rett syndrome. We at Rettsyndrome.org are very proud to have supported this game-changing study, believing that the best is yet to come."

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About Rettsyndrome.org

Rettsyndrome.org is the most comprehensive nonprofit organization dedicated to accelerating research of treatments and a cure for Rett syndrome to accelerate full spectrum research to treat and cure Rett syndrome while empowering the community through knowledge and connectivity.

As the world's leading private funder of Rett syndrome research, Rettsyndrome.org has funded over \$40M 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](http://www.rettsyndrome.org) or call (800) 818-7388 (RETT).