



# Biotech Daily

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## Neuren Tumbles 43% On Acadia \$630m Nth America Trofinetide Licence

Neuren fell as much as 45.3 percent on news that Acadia will pay \$630 million in upfront fees, milestones and royalties for North American rights to trofinetide for neuro indications.

Neuren said that the San Diego, California-based Acadia Pharmaceuticals would pay \$US10 million (\$A13.54 million) upfront and up to \$US455 million (\$A616.2 million) in milestones as well as royalties to licence trofinetide for Rett syndrome, Fragile X and other indications for North America, alone.

Neuren executive chairman Dr Richard Treagus told Biotech Daily the licence covered all indications in North America as well as a first negotiation option for other territories.

Dr Treagus said Neuren was entitled to one third of the market value of any rare paediatric disease priority review voucher, if awarded by the US Food and Drug Administration on approval of a new drug application for trofinetide, as well as full access to all data generated by Acadia for Neuren's applications in other jurisdictions.

Dr Treagus said that FDA priority review vouchers had "recently traded from \$US110 million to \$US245 million".

In a media release, Neuren said it was eligible for tiered, escalating, double-digit percentage royalties on net sales of trofinetide in North America with milestone payments of \$US105 million for Rett syndrome and Fragile X syndrome and up to \$US350 million subject to annual net sales of trofinetide in North America.

The company said Acadia would fund and execute the remaining development for trofinetide in Rett syndrome in North America, except for the completion by Neuren of certain preparatory activities, and Neuren had an obligation not to develop a competing product in indications for which Acadia developed and commercialized trofinetide.

Neuren said that Acadia planned to start a 180-girl, phase III study of trofinetide for Rett syndrome by the end of 2019 following completion of additional manufacturing activities.

The company said the Acadia phase III trial would measure the Rett syndrome behavior questionnaire (RSBQ), a caregiver assessment, and the clinical global impression of improvement (CGI-I) and a physician assessment, as co-primary efficacy endpoints.

Acadia head of research and development Dr Serge Stankovic said that a potential treatment for Rett syndrome was “a perfect fit with Acadia’s mission to develop novel therapies to improve the lives of patients with central nervous system disorders”.

“Today there are no approved treatments for the girls and women suffering from Rett syndrome,” Dr Stankovic said.

Dr Treagus said that Acadia had “a proven record in developing and commercializing medicines in central nervous system disorders”.

“Acadia’s additional capabilities and resources will immediately make a very significant difference, enabling us to advance our shared goal of developing this novel treatment option for Rett syndrome patients,” Dr Treagus said.

Rettsyndrome.org chief science officer Dr Steve Kaminsky said his organization “grateful to Neuren for their dedication to the development of trofinetide”.

“Acadia’s commitment to advance trofinetide to phase III brings us closer to the first potential treatment for Rett syndrome with a drug designed to address the underlying biology and improve the lives of those suffering from the condition,” Dr Kaminsky said.

Neuren said that trofinetide had been granted FDA fast track status and orphan drug designation in the US and Europe.

The company said its phase II, double-blind, placebo-controlled, dose-ranging study in 82 girls aged five to 15 years with Rett syndrome, showed “statistically significant and clinically meaningful improvement” on the Rett syndrome behavior questionnaire and the clinical global impression of improvement measures (BD: March 22, 2017).

Neuren closed down \$1.15 or 43.1 percent to \$1.52 with 3.2 million shares traded.