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Neuren Pharmaceuticals: 'Acadia Phase III Trofinetide Rett Trial Success' - Up 109%

Neuren says that a 187-patient, phase III trial of trofinetide shows statistically significant benefit for Rett syndrome compared to placebo, for both co-primary endpoints.

Neuren said that its North America partner, the San Diego, California-based Acadia Pharmaceuticals, reported "statistically significant improvement over placebo in the Rett syndrome behavior questionnaire ($p = 0.0175$) and the clinical global impression of improvement ($p = 0.0030$).

In 2019, Neuren said Acadia had begun the phase III, 12-week, double-blind, randomized, placebo-controlled, 'Lavender' trial, and previously said that Acadia would pay $630 million in upfront fees, milestones and royalties for North American rights to trofinetide (BD: Aug 7, 2018, Feb 4, Oct 31, 2019).

Neuren said that development and trial costs would be funded by Acadia and Neuren would have free and full access to all data to commercialize outside North America.

In 2020, Neuren said the US Food and Drug Administration had awarded Acadia rare paediatric disease designation for the US study of trofinetide for Rett syndrome, with Acadia eligible for a priority review voucher, with Neuren to receive one third of the market value of the voucher (BD: Mar 4, 2020).

The company said that in 2019, two vouchers were sold for $US105 million ($A159.2 million) and $US95 million ($A144.0 million).

Today, Neuren said it was eligible to receive potential milestone payments of up to $US455 million plus tiered escalating double-digit percentage royalties on net sales of trofinetide in North America, and it would earn revenue over 2022 and 2023 for Rett syndrome in the US alone of $111 million plus double-digit percentage royalties on net sales if a new drug application was approved by the FDA and trofinetide was launched in the US, and it expected to engage commercial partners for Europe and Asia.

The company said that trofinetide met the trial's co-primary efficacy endpoints of statistically significant improvement over placebo in the Rett syndrome behavior questionnaire and the clinical global impression of improvement, as well as key secondary endpoints, including the caregiver scale of ability to communicate.

Neuren said that Acadia planned to conduct a pre-new drug application meeting with the FDA by April 2022 for a new drug application "around mid-year 2022".

An attached media release from Acadia said that discontinuation rates related to treatment emergent adverse events, with 17.2 percent in the trofinetide group compared to 2.1 percent in the placebo group.

Acadia said that the most common adverse events were diarrhoea in which the trofinetide group had an 80.6 percent rate compared to 19.1 percent with placebo, of which 97.3 percent in the trofinetide arm "were characterized as mild-to-moderate" and vomiting which had 26.9 percent with trofinetide compared to 9.6 percent with placebo, "of which 96 percent in the trofinetide arm were characterized as mild-to-moderate".

Acadia said that serious adverse events were observed in 3.2 percent of study participants in both the trofinetide and placebo groups.

Acadia said that patients completing the Lavender study had the opportunity to continue to receive trofinetide in the open-label Lilac and Lilac-2 extension studies, with more than 95 percent of participants who completed the Lavender study electing to roll-over to the Lilac open-label extension study.

Neuren chief executive officer Jon Pilcher said the company was "delighted with these robustly positive results and are now eager to see trofinetide progress through the regulatory approval process".

Neuren climbed as much as 109.4 percent to $3.56, before closing up $1.55 or 91.2 percent at $3.25 with 10.1 million shares traded.