Biotech Daily

Monday March 13, 2023

Daily news on ASX-listed biotechnology companies

Neuren Up 27% On FDA Arcadia Daybue/Trofinetide For Rett Approval

NEUREN PHARMACEUTICALS

Neuren says North America partner Acadia Pharmaceuticals has US Food and Drug Administration approval for Daybue, or trofinetide, for Rett syndrome.

Neuren said that approval was in adults and children two years of age and older.

Biotech Daily believes this is the first FDA approval for an Australian drug since Telix's Illucix for prostate cancer imaging in December 2021 (BD: Jan 16, 2022.

Other recent Australian-developed drugs approved by the FDA include Perth's Peron Institute's casimersen, eteplirsen and golodirsen for Duchenne muscular dystrophy in 2021; Pharmaxis Bronchitol for cystic fibrosis and Hatchtech's Xeglyze for head lice and eggs in 2020, Clinuvel's Scenesse for erythropoietic protoporphyria (EPP) in 2019 and Medicines Development for Global Health (MDGH) moxidectin for river blindness or onchocerciasis in 2018 (BD: Jun 14, 2018; Oct 9, 2019; Aug 3, Nov 2, 2020; Apr 1, 2021).

Today, Neuren said that trofinetide was the "first and only approved treatment for Rett syndrome" and Acadia expected it to be available by the end of April 2023.

The company said the San Diego, California-based Acadia had been awarded a Rare Pediatric Disease Priority Review Voucher with the approval and it would receive $US40 million on the first commercial sale, royalties on net sales, potential sales milestone payments and one third of the Priority Review Voucher, about $US33 million.

Acadia said the approval was supported by the 187-patient, phase III Lavender study which showed statistically significant improvement compared to placebo on both co-primary efficacy endpoints, as measured by the change from baseline in Rett syndrome behavior questionnaire total score (p = 0.018) and the clinical global impression-improvement scale score (p = 0.003), at week 12 (BD: Dec 7, 2021).

Neuren chief executive officer Jon Pilcher said that “many people have shown great determination over the long journey to reach this historic outcome”.

“The greatest has been shown by the Rett syndrome community and I am delighted for them,” Mr Pilcher said.

“For Neuren, this is a transforming milestone that places us in a position to make the most of the opportunities ahead of us, as we work with the communities to make a difference in four other neurodevelopmental disorders,” Mr Pilcher said.

Neuren said that it received a $US10 million milestone from Acadia following the acceptance of the new drug application for review by the FDA and was eligible for royalties on net and milestone payments up-to US$350 million.

The company said it had an exclusive licence with Acadia to develop and commercialize trofinetide for Rett syndrome and other indications in North America and retained all rights to trofinetide for all countries outside North America (BD: Aug 7, 2018).

Neuren said it intended to pursue registration and commercialization of trofinetide through partners and was currently advancing discussions with third parties.

Former seven-year Neuren executive chair, Dr Richard Treagus said that “what makes this accomplishment all the more remarkable is that the Neuren team designed and validated both the endpoint measurement tool for Rett syndrome, at the same time as developing and proving up a novel drug compound”.

“We started with a blank sheet of paper just over 10 years ago and built every aspect in excruciating detail from the ground up,” Dr Treagus said.

“Ultimately the partnership with Acadia in 2018 secured the significant funding pathway for phase III, FDA approval and market access,” Dr Treagus said.

“But congratulations are in order for Prof Margaret Brimble the inventor of the compound and to Jon [Pilcher] for his terrific leadership bringing this home,” Dr Treagus said.

Neuren climbed as much as $2.07 or 27.0 percent to $9.74 before closing up $1.42 or 18.5 percent at $9.09 with 4.2 million shares traded.