



pharmaceuticals

Company profile

Neuren Pharmaceuticals (ASX: NEU)

Neuren has two patented drugs, trofinetide and NNZ-2591, being developed to treat neurological disorders for which currently there are no approved drug therapies. The Phase 3 trial of trofinetide for Rett syndrome will commence in 2019 and Neuren has completed a Phase 2 clinical trial in Fragile X syndrome. Both programs have been granted Fast Track designation by the FDA and Orphan Drug designation in both the US and the EU. Rett syndrome is a debilitating condition that occurs worldwide in approximately one of every 10,000 to 15,000 female births. A genetic mutation causes serious problems in brain function, following apparently normal development for the first six months of life. Neuren recently completed a partnering agreement with ACADIA Pharmaceuticals Inc. for the development and commercialization of trofinetide in North America. ACADIA provides the capabilities, strategic intent and funding required to bring trofinetide to market in the US for Rett syndrome and Fragile X syndrome. ACADIA will fund all development costs and the agreement secures significant participation for Neuren in the future commercial value of trofinetide in North America. Neuren has retained 100% of the value of trofinetide outside North America, including Europe and Japan, with free use of all data and regulatory information generated by ACADIA in the US for registration in other territories.

Neuren receives from ACADIA escalating tiered double-digit percentage royalties on net sales of trofinetide in North America, plus development and sales milestone payments of up to US\$465 million, plus one third of the value of any Rare Pediatric Disease Priority Review Voucher awarded by the FDA (5 vouchers sold for between US\$110 million and US\$150 million in 2017). Following the first payment of US\$10 million from ACADIA, Neuren had \$24.8 million cash at 31 August, enabling it to advance the development of NNZ-2591.

Trofinetide and NNZ-2591 are patented synthetic analogs that replicate the activity of naturally occurring peptides in the brain, but are more stable, orally bioavailable and more readily cross the blood-brain barrier.



Speaker Profile

Jon Pilcher, CFO



Jon has been CFO for 5 years, during which Neuren has progressed to Phase 3 in Rett syndrome and completed a partnering deal for trofinetide in North America. He was previously at Acrux for 11 years, which encompassed the IPO, development to US registration of three products and partnering transactions in the US and Europe. Jon qualified as a Chartered Accountant after completing an honours degree in Biotechnology at Reading University in England. He spent 7 years in the corporate and R&D functions of international pharmaceutical groups Medeva and Celltech (now part of UBC), based in the UK.