

Neuren receives orphan drug designation from the FDA for NNZ-2566 in Fragile X Syndrome

Melbourne, Australia, 30 October 2013: Neuren Pharmaceuticals (ASX: NEU) announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to NNZ-2566 for treatment of Fragile X Syndrome. Orphan drug designation is a special status that the FDA may grant to a drug to treat a rare disease or condition. Orphan drug designation qualifies the sponsor of the drug for seven years of marketing exclusivity following approval as well as various development incentives including waiver of the prescription drug user fee for a marketing application.

Neuren recently announced that its Fragile X Syndrome development programme has received Fast Track designation from the FDA, which is designed to expedite the development and review of important new medicines.

Neuren will commence a Phase 2 double-blind, placebo-controlled trial of NNZ-2566 in Fragile X Syndrome in December 2013. Enrolment is expected to be completed by the end of 2014, with top-line results announced in the first half of 2015. The study is designed to assess the safety, tolerability and efficacy of NNZ-2566 in treating symptoms of Fragile X Syndrome.

Commenting on the FDA's decision, Larry Glass, Neuren's CSO, said: "We are delighted that the FDA has recognised that NNZ-2566 meets the criteria for orphan drug designation. Together with the compelling results in preclinical testing of NNZ-2566, this reinforces our commitment to develop a safe and effective medicine to address the significant unmet medical need of individuals with Fragile X Syndrome."

About NNZ-2566

NNZ-2566 is a synthetic analogue of a naturally occurring neurotrophic peptide derived from IGF-1, a growth factor produced by brain cells. In animal models, NNZ-2566 exhibits a wide range of important effects including inhibiting neuroinflammation, normalising the role of microglia and correcting deficits in synaptic function. In the Fragile X model, these actions resulted in statistically significant improvement in all core anatomic and behavioural features of the disorder that were assessed. NNZ-2566 is being developed both in intravenous and oral formulations for a range of acute and chronic conditions. The intravenous form of NNZ-2566 is presently in a Phase II clinical trial in patients with moderate to severe traumatic brain injury as well as a Phase II trial in Rett Syndrome. Both programs have received Fast Track designation from the US FDA. The company intends to implement a Phase II clinical trial in Fragile X Syndrome and an additional Phase II trial with the oral form of NNZ-2566 in patients with concussion or mild TBI.

About Fragile X Syndrome

Fragile X syndrome is the most common inherited cause of intellectual disability and the most common known cause of autism. It affects 1 out of 4000 males and 1 out of 6-8000 females. Fragile X Syndrome is due to a single gene defect on the X chromosome that impacts the FMRP protein, which is responsible for regulating the synapses of nerve cells. Clinically, Fragile X Syndrome is characterized by intellectual handicap, hyperactivity and attentional problems, autistic symptoms, anxiety, emotional lability and epilepsy. The epilepsy seen in Fragile X Syndrome is most commonly present in childhood, but then gradually improves towards adulthood. Physical features such as prominent ears and jaw, and hyper-extensibility of joints are frequently present but are not diagnostic. Generally, males are more severely affected than females. Currently, there are no medicines approved for the treatment of Fragile X Syndrome.

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a publicly listed biopharmaceutical company focusing on the development of new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. The novel drugs target chronic conditions such as Rett Syndrome and Fragile X Syndrome as well as acute neurological injuries. Neuren presently has a clinical stage molecule, NNZ-2566 in two Phase 2 clinical trials as well as NNZ-2591 in pre-clinical development.

Forward-looking Statements

This ASX-announcement contains forward-looking statements that are subject to risks and uncertainties. Such statements involve known and unknown risks and important factors that may cause the actual results, performance or achievements of Neuren to be materially different from the statements in this announcement.

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