Neuren opens new IND for NNZ-2566 in Fragile X Syndrome

Australia, 9 September 2013: Neuren Pharmaceuticals (ASX:NEU) is pleased to announce that it has received approval from the US Food and Drug Administration (FDA) to conduct a Phase 2 clinical trial of NNZ-2566 in patients with Fragile X Syndrome. Neuren intends to initiate the trial before the end of 2013.

Adolescent and adult males will be enrolled in a double-blind, placebo-controlled study, which will test two different dose levels of NNZ-2566. Preparations for the study, including clinical site qualification and selection, are well underway. Enrolment is expected to be completed by the end of 2014, with top-line results announced in the first half of 2015.

Commenting on the approval of the IND, Larry Glass, Neuren's Chief Science Officer, said: "This is the fourth IND for NNZ-2566, adding to the TBI, concussion and Rett Syndrome INDs. The approval of the IND for Fragile X Syndrome reinforces our strategy to expand the use of NNZ-2566 in both acute and chronic indications. It directly supports our approach of bringing a potentially life-changing medicine to patients and maximising value for our shareholders. It is also a testament to the capabilities and expertise of Neuren's clinical development and regulatory team. We are all encouraged by this latest approval from the FDA and are looking forward to commencing the trial this year."

About NNZ-2566

NNZ-2566 is a synthetic analogue of a naturally occurring neuropeptide derived from IGF-1, a growth factor produced by brain cells. In animal models, NNZ-2566 inhibits neuroinflammation and normalises the function of microglia with consequent improvements in molecular, cellular, anatomic and behavioural outcomes. 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. Neuren currently has operations in New Zealand, Australia and the United States.

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.

For more information, please contact:

Larry Glass, CSO
lglass@neurenpharma.com
Tel: +1 301 941 1830

Dr Richard Treagus, Executive Chairman
rtreagus@neurenpharma.com
Tel: +61 417 520 509