



Neuren completes enrolment of second patient cohort; provides update on NNZ-2566 program

SYDNEY, Australia, 21 November 2011: Neuren Pharmaceuticals Limited (ASX:NEU) announced today that enrolment of the second cohort in the *INTREPID*⁻²⁵⁶⁶ traumatic brain injury (TBI) trial has been completed. Review of safety data by the Data Safety and Monitoring Committee has been finalized and the third cohort has been opened for enrolment. There were no Serious Adverse Events (SAEs) in Cohort 2 patients reported as being drug-related.

As part of Cohort 3, implementation of the protocol approved under Exception from Informed Consent (EFIC) provisions is in progress. EFIC facilitates study execution by allowing enrolment of patients for whom it is not possible to obtain informed consent from a legally authorized representative. EFIC is restricted to situations in which the condition is life-threatening, immediate treatment is required and there is no alternative treatment available. The first phase of EFIC implementation is a program of community consultation and public disclosure at each participating site to inform the community and seek feedback on the trial. This process is well underway.

Neuren is also developing an oral form of NNZ-2566 to treat patients who have had a concussion, a milder type of head injury than the TBI being targeted with the intravenous (IV) form of the drug. Concussions are more than four times as common as moderate or severe TBI, frequently occurring in people participating in sports and as a result of falls and motor vehicle accidents. The oral version of NNZ-2566 is a liquid produced by dissolving the same powder used to make the IV form in a water-based solution. The additional toxicology and pharmacokinetic studies in animals that are required to initiate human trials have been completed and showed the oral form to be safe with minimal side effects. Preparations for a Phase I safety and pharmacokinetic study in healthy volunteers have been finalized and the study to be undertaken in Australia is planned for early 2012. A Phase II trial in concussion patients is expected to start in mid-2012.

In addition to concussion, the Company has begun development of the oral form of NNZ-2566 for Rett Syndrome, a very severe, physically disabling disease and is considered one of the autism spectrum disorders. There is no approved drug for Rett Syndrome which occurs in approximately 1 of 10,000 female children worldwide. Preliminary results with NNZ-2566 in an animal model of Rett Syndrome were promising. A Phase IIa protocol to establish proof of principle in Rett Syndrome patients has been developed. We plan to file an IND for the Rett Syndrome study in the second half of 2012 and to initiate the clinical trial in late 2012. The Company believes that Rett Syndrome will qualify for Orphan Disease and Fast Track designation under US FDA regulations.

Commenting on the NNZ-2566 program, Larry Glass, Neuren's CEO, said: "We are pleased that Cohort 2 was completed successfully and that NNZ-2566 appears to be well-tolerated in TBI patients. We are also gratified that the changes instituted in the study have improved the pace of enrolment and remain confident that enrolment will continue to accelerate in Cohort 3 with the inclusion of female as well as younger and older patients and as EFIC is implemented. Progress with the oral formulation has been excellent and we are excited about the new clinical programs that we believe will significantly increase the value of the NNZ-2566 franchise."

About the Phase II cohorts

The *INTREPID*²⁵⁶⁶ Phase II clinical trial is evaluating the safety and efficacy of intravenous (IV) administration of NNZ-2566 in patients with moderate to severe traumatic brain injury. The drug is administered within 8 hours of injury (within 6 hours under EFIC) via a 10 minute bolus followed by 72 hours of continuous infusion. There are 3 cohorts in the study. All patients receive the same bolus (20 mg/kg/hr) or placebo. The first 30 patients (cohort 1) received a low-dose continuous infusion (1 mg/kg/hr) or placebo. The next 30 patients (cohort 2) received an intermediate dose infusion (3 mg/kg/hr) or placebo. The third cohort will enrol 200 patients who will receive the highest infusion dose (6 mg/kg/hr) or placebo. All doses are intended to produce blood levels of the drug in patients that are comparable to the range in which efficacy was confirmed in animal models of brain injury.

About NNZ-2566

NNZ-2566 is a patented, synthetic analog of the n-terminal tripeptide of IGF-1, a naturally occurring molecule with potent neuroprotective effects in animal models of stroke and head injury. The principal mechanism by which NNZ-2566 exerts its neuroprotective effects is by inhibiting the expression of inflammatory molecules (cytokines) following brain injury. NNZ-2566 also significantly reduces activation of microglia, a type of brain cell involved in the immune response in the central nervous system, but which can result in excessive inflammation following brain injury resulting in additional damage. NNZ-2566 also has been shown to dramatically reduce the incidence of convulsive and non-convulsive seizures in multiple brain injury models. Studies have shown that NNZ-2566 is effective when administered both as an intravenous infusion and in oral form. NNZ-2566 has been in development as a treatment for TBI under a collaborative research and development agreement between Neuren and the US Army since 2004. The US Army has committed approximately US\$23 million to support the NNZ-2566 program.

About Neuren

Neuren Pharmaceuticals is a biopharmaceutical company developing new therapies for brain injury, chronic neurological diseases and cancer. Neuren presently has two clinical-stage molecules, NNZ-2566 and Motiva[®], in Phase II clinical trials largely funded by the US Army and the National Health and Medical Research Council, respectively. Through its subsidiary, Perseis Therapeutics Limited, Neuren is developing monoclonal antibodies against Trefoil Factors 1 and 3, proteins produced by cancer cells that are associated with cancer spread and reduced patient survival.

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