



Neuren (NEU) - ASX Announcement

30 December 2014

Neuren submits Orphan Drug and Breakthrough Therapy applications to the FDA

Melbourne, Australia, 30 December 2014: Neuren Pharmaceuticals (ASX: NEU) today announced that it has submitted applications to the US Food and Drug Administration (FDA) for Orphan Drug designation and Breakthrough Therapy designation for NNZ-2566 in Rett syndrome.

Orphan Drug designation is a special status that the FDA may grant to a drug to treat a rare disease or condition. Amongst other incentives, Orphan Drug designation qualifies the sponsor of the drug for seven years of marketing exclusivity and various development incentives including waiver of the prescription drug user fee for a marketing application. The FDA has previously granted Orphan Drug designation to Neuren for NNZ-2566 in Fragile X syndrome.

Breakthrough Therapy is an expedited program in the United States, intended to streamline drug development and regulatory review of innovative new medicines that address unmet medical needs for serious diseases or conditions. The criteria for Breakthrough Therapy require preliminary clinical evidence indicating that the drug may demonstrate a substantial improvement over existing therapies on at least one clinically significant endpoint. Breakthrough Therapy designation conveys all of the Fast Track program features, as well as a commitment that FDA will work closely with the sponsor on an efficient drug development program. FDA's target timeline for responding to an application for Breakthrough Therapy designation is within 60 days.

"Neuren ends 2014 in a very strong position following the results from our first Phase 2 trial in Rett syndrome", commented Neuren Executive Chairman, Richard Treagus. "The results exceeded our expectations and have led to several enquiries from international pharmaceutical companies. Our objective for 2015 is to move NNZ-2566 through development and towards marketing approval as quickly and efficiently as possible. Neuren remains well funded, with cash reserves of approximately \$21 million."

Neuren also provided an update on its four clinical development programs for NNZ-2566.

Rett syndrome

In November 2014, Neuren announced top-line results from its Phase 2 clinical trial in Rett syndrome, which successfully demonstrated clinical benefit from treatment with NNZ-2566. The benefit observed in the trial encompassed many of the core symptoms of Rett syndrome and was observed in both clinician and caregiver assessments. Neuren has now submitted applications to the FDA for Orphan Drug designation and Breakthrough Therapy designation. Neuren expects to meet with the FDA in the



first half of 2015 to discuss the remaining requirements for the development of NNZ-2566 in Rett syndrome.

Fragile X syndrome

To date, 22 subjects have commenced treatment in Neuren's Phase 2 trial of NNZ-2566 in Fragile X syndrome, 19 of which have already completed. The trial represents a significant commitment for families, with only 2 subjects entering the trial during the US holiday period in November and December. Neuren is taking a number of steps to accelerate enrolment into the trial, including working closely with advocacy organisations to ensure that the recent results of the trial of NNZ-2566 in Rett syndrome are widely communicated. The number of trial sites enrolling will increase from 6 to 9 in January 2015 and further sites will be added to expand the geographical coverage in the United States. Neuren is also currently investigating the feasibility of extending the trial to include sites in Australia. Top-line results from the trial that were targeted for the second quarter of 2015, are now expected in the second half of 2015.

Brain injury

Enrolment in Neuren's Phase 2 trial of NNZ-2566 in moderate-to-severe traumatic brain injury (the "INTREPID" trial) has risen to 195 subjects. 16 trial sites in the United States are now active, with 2 more due to commence early in 2015. Neuren expects top-line results to be available in the second half of 2015.

Neuren's fourth Phase 2 trial of NNZ-2566, in concussion (mild traumatic brain injury), commenced in September 2014 with the US Army's 82nd Airborne Division at Fort Bragg in North Carolina. In the first half of 2015, the trial will be expanded to include civilian trial sites in order to accelerate enrolment of the targeted 132 subjects. Top-line results from the trial are expected in the second half of 2015.

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, normalizing the role of microglia and correcting deficits in synaptic function. 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 2 clinical trial in patients with moderate to severe traumatic brain injury. The oral form of NNZ-2566 is in Phase 2 development in Rett syndrome, Fragile X syndrome and mild traumatic brain injury (concussion). Three programs have received Fast Track designation from the US FDA and the Fragile X syndrome program has also received Orphan Drug designation.



About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. The novel drugs target chronic conditions as well as acute neurological injuries. Neuren presently has a clinical stage molecule, NNZ-2566 in 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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