Neuren (NEU) - ASX Announcement **26 August 2020**

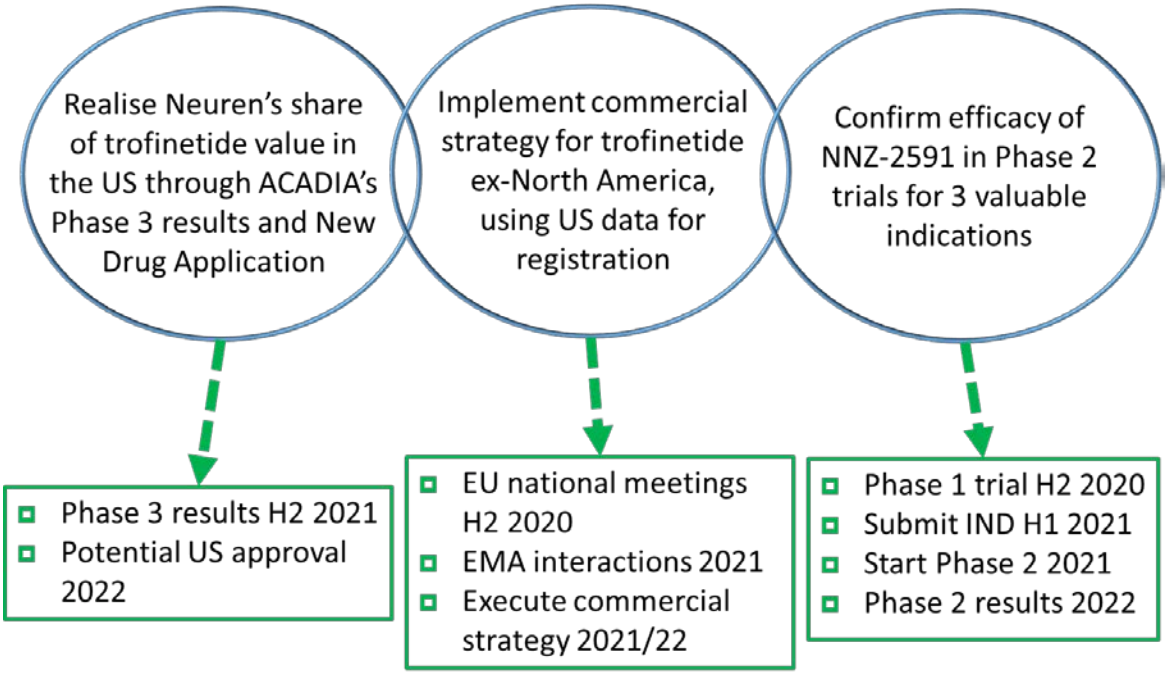
Half-Year Shareholder Update

Dear Neuren shareholders,

Today we filed our 2020 half-year financial report, which showed cash reserves at 30 June of $9 million, which increased to $28 million in July following the capital raising at $1.40 per share. The capital raising was very important – without it NNZ-2591 would have remained an interesting asset without the validation of its value that proof of concept data in patients provides. It also enabled us to increase institutional shareholdings to approximately one third of the share register, up from only 5% six months ago. We are pleased that new institutional investors came from four different countries, which is a positive step in the evolution of our share register.

The fact that we are now able to pursue Phase 2 data for NNZ-2591 in multiple indications is very important for Neuren and investors from the point of view of both risk and opportunity. For risk, it more than doubles our number of "shots on goal" and for opportunity it transforms the potential "upside", adding new target markets with four times as many patients as Rett syndrome.

As shown in the familiar graphic below, we now have three large value drivers that can crystallise in 2021 and 2022 and we are very focused on executing each step towards achieving them.



The first large value driver is to deliver the milestone payments and royalties from the ACADIA deal. The Rett syndrome LAVENDER and LILAC trials are continuing in the United States and ACADIA in its recent Q2 Earnings Call re-confirmed that the results of LAVENDER are expected in the second half of 2021. It is

very frustrating for all stakeholders, not least the patients and their families, that the unprecedented COVID-19 pandemic arrived during this highly anticipated trial, causing travel restrictions and safety risks. After ending a 3 month pause in enrolment in June 2020, I am confident that ACADIA is taking all appropriate measures, working with each trial site individually to get the best outcome. I also remain confident about the potential impact of trofinetide on this devastating condition, having experienced the two clinical trials that Neuren conducted in girls and women with Rett syndrome.

With positive results from LAVENDER, we expect that Neuren will have multiple commercial options outside North America, which is our second large value driver. These options will include partnering by territory, a deal with a single partner and/or commercializing directly in some countries. To further our position in the meantime, we have started discussions with national regulatory authorities in Europe. The purpose of these non-binding meetings is to gain their support and their advice to optimize our approach to the European Medicines Agency (EMA) under the centralized procedure that is required for Orphan Drugs.

For the third large value driver, which is to confirm in patients the outstanding efficacy results seen for NNZ-2591 in animal models of all three disorders, we are working in parallel on three elements of an Investigational New Drug (IND) application to the FDA. We are nearing completion of the necessary non-clinical safety studies, the Phase 1 trial to characterize safety and PK in healthy volunteers is continuing in Western Australia, and we are evaluating alternative designs for the Phase 2 trials. We will provide updates on all of these in due course.

I will conclude by noting that there are very few companies on the ASX with an ongoing single Phase 3 trial fully funded by a partner and four other programs either in or soon to start Phase 2. All five are serious conditions in children with no approved medicines. We are aiming to improve the lives of families impacted by all these conditions and I am grateful for your support as we strive step-by-step to achieve it.

Jon Pilcher, CEO

About Neuren

Neuren is developing new therapies for neurodevelopmental disorders with high unmet need, utilizing synthetic analogs of neurotrophic peptides that occur naturally in the brain. Trofinetide is currently in a Phase 3 clinical trial for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome.

The programs have each received Fast Track designation by the US Food and Drug Administration and Orphan Drug designation in both the United States and the European Union. Neuren has granted an exclusive license to ACADIA Pharmaceuticals Inc. for the development and commercialization of trofinetide in North America, whilst retaining all rights outside North America. Neuren is advancing the development of NNZ-2591 for Phelan-McDermid, Angelman and Pitt Hopkins syndromes, each of which has received Orphan Drug designation in the United States.

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ASX Listing Rules information

This announcement was authorized to be given to the ASX by the board of directors of Neuren Pharmaceuticals Limited, Suite 201, 697 Burke Road, Camberwell, VIC 3124

Forward-looking Statements

This 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.