

## **MND Trial Completes Enrolment of Cohort 2 and Prepares for Interim Analysis**

- **PharmAust has completed enrolment of all 12 patients in Cohorts 1 and 2 for the MND clinical trial**
- **On completion of dosing of the last patient of Cohort 2, an interim analysis will commence to evaluate potential indicatory changes in biomarkers and pharmacodynamics**
- **Mid-point outcomes in the MND trial will include information on absorption and pharmacokinetics of MPL**
- **An interim analysis of target engagement and treatment-related changes in biomarkers is expected**
- **PharmAust will continue with MPL dose escalation for Cohorts 3 and 4 to determine the optimum dose for a Phase 2 trial**
- **Indicatory evidence for MND severity and progression during the trial will also be examined**
- **PharmAust expects to proceed to Phase 2 with favourable efficacy biomarker results under the interim analysis**

**30 March 2023 – Perth, Australia:** PharmAust Limited (ASX: PAA & PAAO), a clinical-stage biotechnology company, has completed enrolment of the second cohort of six patients in its Phase 1/2 clinical trial of its lead drug candidate monepantel (MPL) in Motor Neurone Disease/Amyotrophic Lateral Sclerosis (MND/ALS).

The patients are enrolled at two sites: Calvary Health Care Bethlehem, Statewide Progressive Neurological Disease Service, Caulfield South in Victoria and The Centre for Motor Neurone Disease Research, Faculty of Medicine and Health Research Macquarie University in NSW.

The Phase 1/2 clinical study aims to determine the tolerability, safety, pharmacokinetics and preliminary efficacy of oral MPL in individuals living with MND. The trial is open label and comprises four cohorts with escalating MPL doses.

So far the MPL tablets have been well tolerated by patients in the first and second cohorts of the trial and the Safety Monitoring Committee will continue the assessment of safety and efficacy for each dosage.

### **Interim analysis**

As announced on 2 March 2023, the Principal Investigator recommended undertaking an interim analysis of preliminary biomarkers and efficacy markers on completion of dosing of the last patient of Cohort 2.

Treatment-related changes from baseline in this safety, tolerability, pharmacokinetic and preliminary, efficacy study will include an analysis of functional rating scales, quality of life and cognitive assessment. Further, prognostic indicators and several disease-related biomarkers will be measured.

PharmAust will also continue with the MPL dose escalation for Cohorts 3 and 4 during the interim trial analysis to determine the optimum dose level for the Phase 2 trial.

Subject to Safety Committee reviews, the patients from Cohort 1 will move to Cohort 3 and the patients from Cohort 2 will move to Cohort 4.

### **MPL a promising treatment for MND**

According to the International Alliance of ALS/MND Associations, MND affects over 350,000 people globally and kills more than 100,000 people every year. The disease is invariably fatal with the average life expectancy of someone who has MND being around 27 months. The MND/ALS addressable market is US\$3.6Bn per annum with Riluzole already reaching ~US\$1Bn annual sales.

The disease is progressive, meaning the symptoms get worse over time. MND has no cure and there is no effective treatment to reverse its progression. PharmAust notes patients have been dosed with MPL for up to six months in the clinical trial with no signs of material adverse events and are “stable”.

PharmAust demonstrated in its preclinical programs that MPL has the potential to activate molecular pathways relevant to the treatment of MND. MPL could potentially reduce the rate of degeneration and loss of motor neurons in the anterior horns and motor nuclei of the brainstem. There are also a number of surrogate clinical endpoints to be determined during the trial. PharmAust has developed and manufactured a bespoke MPL tablet for the trial.

With success in the clinic PharmAust hopes that in due course MPL could receive orphan drug designation by the TGA and FDA for motor neurone disease. Such designations come with financial and supportive benefits and this opportunity is being evaluated by PAA.

The Phase1/2 study is being funded by a commitment of \$881,085 by FightMND, the largest independent funder of MND research in Australia.

This announcement is authorised by the Board.

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### **About PharmAust Limited:**

PharmAust Limited is listed on the Australian Securities Exchange (PAA) and the Frankfurt Stock Exchange (ECQ). PAA is a clinical-stage company developing therapeutics for both humans and animals. The company specialises in repurposing marketed drugs lowering the risks and costs of development. These efforts are supported by PAA's subsidiary, Epichem, a highly successful contract medicinal chemistry company that generated \$3.4 million in sales of goods & services in FY 2022.

PAA's lead drug candidate is monepantel (MPL), a novel, a potent and safe inhibitor of the mTOR pathway – a pathway having key influences in cancer growth and neurodegenerative diseases. MPL has been evaluated in Phase 1 clinical trials in humans and Phase 2 clinical trials in dogs. MPL treatment was well-tolerated in humans, demonstrating preliminary evidence of anticancer activity. MPL showed objective anticancer activity in dogs. PAA is uniquely positioned to commercialise MPL for treating human and veterinary cancers and neurodegenerative diseases as it advances a reformulated version of this drug through Phase 1 and 2 clinical trials.