First Patient of Cohort 2 Dosed in MND Phase 1/2 Trial

- PharmAust's escalating dosage regimen for the evaluation of Monepantel (MPL) in MND has now enrolled its first patient of Cohort 2 who will receive 4 mg/kg MPL in line with the protocol
- The patient will be receiving twice the corresponding dosage received by patients in Cohort 1
- The outcome of this rising dose study will be a key determinant in proceeding to a phase 2 trial in MND
- All initial six patients in Cohort 1 elected to continue on MPL with the first patient now surpassing 140 days of MPL treatment
- Continued Safety, pharmacokinetics and efficacy data from the trial will also be used to facilitate a Phase 2 study in COVID-19 patients

20th February 2023 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAO), a clinical-stage biotechnology company, is pleased to report the first patient of the second cohort has been dosed in its Phase 1/2 Trial testing the effects of monepantel (MPL) in individuals living with motor neurone disease (MND).

MPL was well tolerated by all MND patients at the first dosing level and pharmacokinetics (PK) confirmed drug absorption. PharmAust will continue to supply MPL tablets to all six patients in Cohort 1 that elected to remain on the treatment. The first patient to be dosed has now surpassed 140 days of MPL treatment with no material safety issues or Serious Adverse Events (SAEs).

Patients from Cohort 1 will continue to be monitored for cognitive function with ALSFRS-R (ALS Functional Rating Scale – Revised) being collected monthly. At the end of each dosing visit, all 3 scales are collected; ALSFRS-R, ALSQOL (Quality of Life Assessment Questionnaire) and ECAS (Edinburgh Cognitive and Behavioural ALS Screen).

Progression of the trial with concomitant escalation of MPL dosing is consistent with the good safety profile during the Cohort 1 stage of the study and that all of the Cohort 1 patients have elected to remain on the drug. Furthermore, the Safety Monitoring Committee has reviewed the safety data and pharmacokinetics from the first dosage level and authorised progression of the trial to the Cohort 2 stage.

PharmAust will now focus on enrolling a further five patients to complete the Cohort 2 group of six patients in its Phase 1/2 clinical trial. The outcome of this rising dose study will be a key determinant in proceeding to a Phase 2 trial in MND.

Continued safety and efficacy data from the trial will also be used to facilitate a Phase 2 study in COVID-19 patients.

PharmAust previously announced it received a funding commitment of A$881,085 for a Phase 1/2 trial examining the effects of MPL in MND, otherwise known as Lou Gehrig's disease or Amyotrophic Lateral Sclerosis (ALS). These funds were granted by FightMND, the largest independent funder of MND

research in Australia. The trial is overseen by Dr Susan Mathers of Calvary Health Care, Bethlehem, Statewide Progressive Neurological Disease Service, Melbourne and will include a second trial site headed by Professor Dominic Rowe of the Centre for Motor Neurone Disease Research Faculty of Medicine and Health Research at Macquarie University, Sydney. The funding agreement provides that PharmAust shall own all intellectual property generated from the trial.

The phase 1/2 clinical study is determining the tolerability, safety, pharmacokinetics and preliminary efficacy of oral MPL in MND sufferers. The trial is open label and comprises a four week escalating dose of MPL.

The following dose level and cumulative data points (where available) will be reviewed as soon as possible for:

- Serious Adverse Events (SAEs)
- Adverse Events (AEs)
- Safety blood results (haematology, chemistry, urinalysis)
- ECG
- Vital signs including temperature, blood pressure, and pulse
- Pharmacokinetic results (PK) and Cerebrospinal Fluid (CSF)

The progressive elevation of MPL levels, as we progress the pharmacokinetic evaluation in MND, will be indicative of the safe dosing levels for our planned COVID-19 trial. In the current trial, levels of MPL are determined in serum after dosing over a 28-day period.

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 just around 27 months. The MND/ALS addressable market is US$3.6Bn per annum with Riluzole already reaching ~US$1Bn in annual sales.

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 monepantel 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 the indication of motor neurone disease. Such designations come with a number of financial and supportive benefits and this opportunity is currently being evaluated by PAA.

This announcement is authorised by the Board.

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About PharmAust Limited:

PharmAust Limited is listed on the Australian Securities Exchange (code: PAA) and the Frankfurt Stock Exchange (code: 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 which generated $3.4 million in sales of goods & services in FY 2022.

PAA's lead drug candidate is monepantel (MPL), a novel, 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 demonstrated objective anticancer activity in dogs. PAA is uniquely positioned to commercialise MPL for treatment of human and veterinary cancers as well as neurodegenerative disease as it advances a reformulated version of this drug through Phase 1 and 2 clinical trials.