PharmAust Phase 1/2 Motor Neurone Disease Clinical Trial Successfully Completes First Patient Cohort

- Monepantel (MPL) was well tolerated by all MND patients at the first dosing level
- All six patients in cohort 1 have elected to continue on MPL treatment
- Subject to satisfying safety, progressive cohorts will receive escalating doses of MPL tablets
- Patient recruitment at the next dosing level expected to begin later this month
- PharmAust will continue to evaluate safety and efficacy in MND patients treated with rising dose levels of MPL that may enable progress to a Phase 2 study in MND
- Safety and efficacy data from the trial will also be used to facilitate a Phase 2 study in COVID-19 patients

6 January 2023 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAO), a clinical-stage biotechnology company, has successfully complete its first 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).

Having announced completion of patient recruitment for treatment level 1 (ASX announcement 1 December 2022), PharmAust has now successfully completed the day 29 dosing of the final patient in the first cohort. The patients were enrolled at two sites: Calvary Health Care Bethlehem, Parkdale, Victoria and the Centre for Motor Neurone Disease Research, Faculty of Medicine and Health Research Macquarie University, Sydney.

Importantly, all of the six patients in this first cohort have elected to continue on MPL treatment.

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 MPL tablets have been well tolerated by patients in the first cohort and the Safety Monitoring Committee will review data from each dosage level for safety and pharmacokinetics.

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 trial. In the current trial, levels of MPL are determined in serum after dosing over a 28-day period.

Patient recruitment at the next dosing level of MPL is expected to begin later this month.

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.

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

With success in the clinic PharmAust hopes that in due course MPL could receive orphan drug designation by the FDA for the indication of motor neurone disease. Such designations come with a number of financial and supportive benefits.

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.