MND Trial Completes Enrolment of First Patient Cohort

- PharmAust has completed enrolment of the first cohort of six MND patients
- At the first treatment level, MPL tablets have been well tolerated
- Subject to satisfying safety, progressive cohorts will receive escalating oral treatment doses of monepantel (MPL) tablets
- Patient recruitment at the next dosing level of MPL is expected to begin in the new year
- PharmAust will continue to evaluate safety and efficacy in MND patients treated with rising dose levels of MPL that will enable the Company to progress to a phase 2 study in MND
- Safety and Efficacy data from the MND trial will facilitate a phase 2 study in COVID 19 patients

2 December 2022 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAO), a clinical-stage biotechnology company, provides an update on its Phase 1/2 clinical trial of its lead drug candidate monepantel (MPL) in Motor Neurone Disease/Amyotrophic Lateral Sclerosis (MND/ALS).

PharmAust, the trial sponsor, is pleased to advise that the trial has completed enrolment of the first cohort of six patients. The patients are 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, New South Wales.

The final patient for the cohort was dosed yesterday and replaces a patient who was excluded as they failed to comply with enrolment requirements.

The phase 1/2 clinical study is aimed at determining the tolerability, safety, pharmacokinetics and preliminary efficacy of oral MPL in individuals living with MND. 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 of the trial and the Safety Monitoring Committee will review data from each Dosage Level for continued safety and pharmacokinetic data up to cycle.

The following dose level and cumulative data points (where available) will be reviewed as soon as possible (approximately 19 working days) after the last participant in the cohort completes Day 29 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 CSF)

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

MND Webinar Recording

PharmAust recently held a special webinar on the clinical trial testing of monepantel in patients living with MND. Principal Investigator Dr Sue Mathers shared her expertise on the disease and insight on monepantel and its unique potential to slow the progression of MND. Despite medical advancements, MND remains a terminal illness with no effective treatment or cure.

Dr Rebecca Sheean, Research Director of FightMND, also joined the panel to share the story of FightMND's search for a cure for MND, including its A$881,085 grant investment in PharmAust's MPL trial.

A recording of the webinar is available on the PharmAust website at the following link –

https://www.pharmaust.com/phamaust-webinar/

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.