PharmAust Progresses to Elevated Doses of MPL in Motor Neurone Disease Patients

- Trial Safety Committee review of the first Cohort clears increased doses of Monepantel (MPL) tablets
- MPL well tolerated by all MND patients at the first dosing level and pharmacokinetics (PK) confirmed drug absorption
- No reported safety issues or Serious Adverse Events (SAEs)
- Patient recruitment at the next dosing level commenced with four new patients undergoing screening
- All initial six patients in Cohort 1 elected to continue on MPL treatment

20 January 2023 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAO), a clinical-stage biotechnology company evaluating the use of Monepantel (MPL) in Motor Neurone Disease (MND), is pleased to announce Trial Safety Committee approval to progress with an escalation of the MPL tablet dosing for MND patients.

The trial patients living with MND/Amyotrophic Lateral Sclerosis (MND/ALS) were evaluated for adverse events and pharmacokinetic information of MPL absorption. The Trial Safety Committee confirmed there were no reported safety issues or SAEs. The PK data also 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 trial is open label and comprises a four-week escalating dose of MPL.

Patient recruitment at the next MPL dosing level has commenced with four new patients currently undergoing screening.

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 average MND life expectancy of about 27 months. The MND/ALS addressable market is US$3.6Bn per annum. While there is no cure for MND/ALS, Riluzole is the only drug licensed for treatment with ~US$1Bn 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 MPL tablet for the trial.

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

With success in the clinic PharmAust is hopeful MPL could receive orphan drug designation by the US FDA for MND. Such designations come with 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.