Progress update on Phase 1/2 MND interim analysis

- PharmAust confirms that all blood samples from the interim analysis have been collected and submitted for analysis
- Analysis of how the MPL is absorbed, distributed, metabolised and eliminated by the body are expected within the week
- An analysis evaluating changes in biomarkers and pharmacodynamics is still pending
- PharmAust will continue with MPL dose escalation for Cohorts 3 and 4 to determine the optimum dose for a Phase 2 trial
- A favourable interim analysis will allow PharmAust to prepare for a Phase 2 MND clinical trial and a Human Cancer Phase 2 clinical trial

15 May 2023 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAO), a clinical-stage biotechnology company, provides a progress update on its Phase 1/2 clinical trial of its lead drug candidate monepantel (MPL) in Motor Neurone Disease/Amyotrophic Lateral Sclerosis (MND/ALS).

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.

PharmAust confirms that all blood samples from the interim analysis have been collected from all 12 patients in Cohorts 1 and 2 and have been submitted for analysis. Results will be announced to the market as they are received. The Company anticipates the pharmacokinetics study results will be available within the week.

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 a Phase 2 trial.

Subject to approval at the next Safety Committee meeting to be held on 18 May 2023, the patients from Cohort 1 will be escalated to Cohort 3. Following further approvals, those patients from Cohort 2 will move to Cohort 4.

The Phase 1/2 MND trial is on track to be completed in Q3 of CY 2023.

MPL is 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 yearly. The disease is invariably fatal, with the average life expectancy of someone with 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 no effective treatment to reverse its progression. PharmAust notes that five patients have surpassed the 6-month mark on MPL without any safety issues, and one patient appears “stable”.

PharmAust demonstrated in its preclinical programs that MPL has the potential to activate molecular pathways relevant to the treatment of MND. MPL could reduce the rate of degeneration and loss of motor neurons in the brainstem's anterior horns and motor nuclei. There are also several 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 MPL could receive orphan drug designation by the TGA and FDA for motor neurone disease. Such designations come with financial and supportive benefits and PAA is evaluating this opportunity.

The Phase 1/2 study is being funded by a commitment of $881,085 by FightMND, Australia's largest independent funder of MND research.

The Board authorises this announcement.

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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.