Interim analysis recommended in MND trial

- **Protocol amendment in MND trial to evaluate potential indicatory changes in biomarkers and pharmacodynamics**
- **Mid-point outcomes resulting from the protocol amendment in the MND trial will include information on absorption and pharmacokinetics of MPL in patients**
- **An interim analysis of target engagement and treatment-related changes in biomarkers is expected, following review after completion of dosing of Cohort 2**
- **PharmAust will continue with the MPL dose escalation for Cohorts level 3 and 4 to determine the optimum dose level for a Phase 2 trial**
- **Emerging indicatory evidence for MND severity and progression during the course of the trial will also be examined**

2 March 2023 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAO), a clinical-stage biotechnology company evaluating the use of Monepantel (MPL) in cancer and motor neurone disease (MND), is pleased to announce it will undertake an interim analysis of preliminary biomarkers and efficacy markers on completion of dosing of the last patient of Cohort 2 in its MND trial.

This interim analysis constitutes an approved protocol amendment to the initial approved protocol as recommended by the Principal Investigator.

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. Furthermore, prognostic indicators and several disease-related biomarkers will be measured.

Following this protocol amendment, PharmAust will continue with the MPL dose escalation for Cohorts level 3 and then 4 (subject to Safety Committee reviews).

This process may facilitate determining the optimum dose level or range for a Phase 2 trial.

PharmAust Executive Chairman, Dr Roger Aston commented:

“Subject to trial outcomes under this protocol amendment, PharmAust expects to prepare the ground for the MND trial to be expedited into Phase 2. The protocol amendment and interim analysis has been recommended by the Principal Investigator.

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 the Phase 2 trial.”

PharmAust acknowledges grant funding from the FightMND charity to support this trial.

The Board authorises this announcement.

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About PharmAust Limited:

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

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