Motor Neurone Disease Phase 1/2 Clinical Trial Update

- **MPL well tolerated by all MND patients at the first two dosing levels**
- **No reported safety issues or Serious Adverse Events (SAEs)**
- **Pharmacokinetic (PK) analysis underway**

22 May 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 provide an update on the Motor Neurone Phase 1/2 Clinical Trial.

The trial patients living with MND/Amyotrophic Lateral Sclerosis (MND/ALS) were evaluated for adverse events. The Trial Safety Committee confirmed there were no reported safety issues or SAEs.

Subject to PK data confirming absorption as it did for Cohort 1, all six patients from Cohort 1 will be elevated to Cohort 3 and will receive an increased dosage. The trial is open label and comprises a four-week escalating dose of MPL. The Phase 1/2 MND trial is on track to be completed in Q3 of 2023.

Progress update on Phase 1/2 MND interim analysis

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 pharmacokinetics study results, which were received last week on time, will be formally interpreted by a pharmacologist and biostatistician and will be announced imminently. An analysis evaluating changes in biomarkers and pharmacodynamics is still pending.

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.

Enquiries:

Anusha Aubert
Investor Relations
investorenquiries@pharmaust.com

P +61 (8) 9202 6814
F +61 (8) 9467 6111
W www.pharmaust.com

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.