Phase 1/2 MND Trial Successfully Completes Third Patient Cohort

- All MND participants have well-tolerated Monepantel (MPL) at the first, second and third dosing levels
- All trial participants have elected to continue on MPL treatment
- Five participants have now surpassed the 9-month-mark on MPL without any safety issues
- Subject to Safety Committee approval, PharmAust will continue with MPL dose escalation for Cohort 4 to determine the optimum dose
- Safety and efficacy data from the trial will also guide other human Phase 2 clinical trials

28 July 2023 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAO), a clinical-stage biotechnology company, has completed its third cohort of six participants in its Phase 1/2 clinical trial of its lead drug candidate monepantel (MPL) in Motor Neurone Disease/Amyotrophic Lateral Sclerosis (MND/ALS).

Having announced the completion of recruitment for treatment level 3 (refer ASX announcement 27 June 2023), PharmAust has completed the day 28 dosing of the final patient in the third cohort.

Importantly, all participants from Cohorts 1, 2 and 3 have elected to continue on MPL treatment.

All participants have tolerated the MPL tablets well, and the Safety Monitoring Committee will review data from each dosage level for safety and pharmacokinetic effects.

The Phase 1/2 clinical study is determining the tolerability, safety, pharmacokinetics and preliminary efficacy of oral MPL in patients living with MND. The trial is open-label and comprises a four week escalating dose of MPL. The patients were enrolled at two sites: Calvary Health Care Bethlehem, Statewide Progressive Neurological Disease Service, Caulfield South and The Centre for Motor Neurone Disease Research, Faculty of Medicine and Health Research Macquarie University, Sydney.

Neurofilament Light Chain results due early August 2023

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.

So far, there is very encouraging data coming from the interim analysis. MPL is proving itself with every biomarker having pointed to a benefit.

The results of the highly specialised testing of Neurofilament Light Chain (NfL) are expected in the coming weeks. The most recent advice is that additional replacement parts for the Simoa machine have been received from the US and an engineer from Melbourne is due to install next week. If the Simoa machine is still un-operational, PharmAust has identified another laboratory who will ensure the NfL assay results can be processed in a similar timeline.

MPL 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 every year. The disease is invariably fatal with the average life expectancy of someone who has 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 there is no effective treatment to reverse its progression. PharmAust notes patients have been dosed with MPL for up to 9 months in the clinical trial with no signs of material adverse events and appear “stable”.

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.

With success in the clinic PharmAust hopes that in due course MPL could receive orphan drug designation by the TGA and FDA for motor neurone disease. Such designations come with financial and supportive benefits and this opportunity is being evaluated by PAA.

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

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.