Final MND Patient in Cohort 4 Successfully Completes Dosing

Highlights:

- **All patients in Cohort 4 have successfully completed dosing in the Phase 1 MEND Study of monepantel in patients with motor neurone disease**
- **Treatment has been well tolerated even after escalation to Cohort 4 and over 12 months of continuous treatment**
- **Patients have one last follow-up visit to attend before the study completes and the data collation process begins verification and finalisation for analysis and release of top-line data in Q1CY24**
- **Patients have elected to continue treatment with monepantel under a compassionate use program and will be eligible to enrol in an open label extension study expected to commence very early in CY 2024**
- **Data from the Phase 1 MEND Study will be used to support an Orphan Drug Designation application and to open an IND with the US FDA to commence a Phase 2 Study in H1CY24**

13 October 2023 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAO) (Company), a clinical-stage biotechnology company, is pleased to announce that all patients in Cohort 4 have successfully completed dosing in its Phase 1 MEND Study of monepantel (MPL) in patients with motor neurone disease (MND/ALS). Data from the Phase 1 MEND Study will be used to support an Orphan Drug Designation (ODD) and to open an Investigational New Drug (IND) application with the United States (US) Food and Drug Administration (FDA) to commence a Phase 2 Study in H1CY24.

PharmAust Chief Executive Officer Dr Michael Thurn commented: "We are extremely pleased with how the study has progressed over the last year. It's an exciting milestone to reach knowing that some patients have been receiving treatment with monepantel for over a year now and patients have elected to continue treatment with monepantel under a compassionate use program.

Patients will also be eligible to roll-over into an Open Label Extension (OLE) Study that is currently in the final stages of preparation for submission to the Human Research Ethics Committee for approval. The OLE Study is expected to begin very early in the New Year and will generate valuable safety and efficacy data for a further 12 months.

This is a fantastic outcome for the Company and for the patients living with this severely debilitating and ultimately fatal disease."

About the Phase 1 MEND Study

The Phase 1 MEND Study is an open label, multicentre study involving 12 patients with MND/ALS with the goal of determining the recommended Phase 2 dose based on safety and preliminary efficacy. The study design (See Figure 1) involves 2 Groups of 6 patients with each Group progressively receiving higher dose levels of MPL in a staggered design approach over time. Progression to a new dose level of MPL was subject to meeting set safety criteria governed by a Safety Monitoring Committee.

During the study, safety and tolerability, pharmacokinetic (MPL and its metabolite MPL sulfone in plasma and cerebrospinal fluid) and pharmacodynamic (p-RPS6KB1 and p-EIF4EBP1 peripheral blood mononuclear cells) parameters, and preliminary efficacy (ALS Functional Rating Scale–Revised,

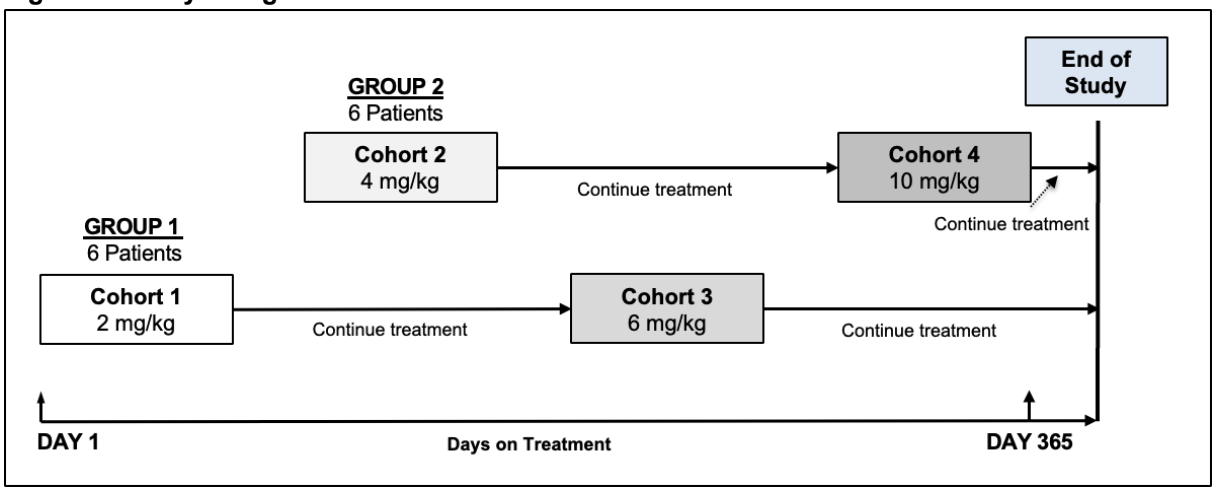
ALS Quality of Life Questionnaire, Edinburgh Cognitive and Behavioural ALS Screen, slow vital capacity and 3 Tesla MRI diffusion kurtosis imaging) and biomarkers (serum neurofilament /light chain, cerebrospinal fluid neurofilament/light chain and urinary p75 levels). measures will be assessed.

The last patient in Cohort 4, the final dose level (10 mg/kg body weight) has now successfully completed dosing. Patients have one last follow-up visit to attend before the study completes and the data collation process begins verification and finalisation for analysis and release of top-line data in Q1CY24.

MND/ALS is invariably fatal with the average life expectancy of someone with MND/ALS being around 27 months. Independent studies have shown that one-third of patients die within 12 months after the first diagnosis. Encouragingly, some patients have been treated with MPL for over 12 months now, no patients have withdrawn from the study, there have been no deaths or significant adverse events.

The study is supported by a drug development grant of $881,085 from FightMND, Australia's largest independent non-for-profit organisation for MND research.

Figure 1: Study Design



Principal Investigator Assoc. Prof Susan Mathers commented: "All of the participants have tolerated the study drug very well with no safety issues, despite a year of treatment. As investigators, we are very grateful for the enthusiastic support and commitment from our patients and their families. Like them, we eagerly await the study results.

Future Directions

Following the success of the Phase 1 MEND Study, PharmAust is currently finalising the protocol and Human Research Ethics Committee (HREC) submission documentation for an Open Label Extension (OLE) Study that allows the 12 patients currently receiving treatment with monepantel to continue treatments for a further 12 months. The OLE Study is expected to start very early in the New Year following HREC approval and will generate valuable safety and efficacy data. In parallel the Company plans to apply for an Orphan Drug Designation and open an IND application with the US FDA to commence a Phase 2 Study for MND/ALS in H1CY24.

Australia and New Zealand MND Research Symposium 2023

PharmAust is a proud Silver Sponsor of the 2nd Australian and New Zealand MND Research Symposium to be held in Wollongong, NSW on Friday 17th and Saturday 18th November. Dr Thurn and Assoc. Prof Mathers will be attending the Symposium.

This announcement is authorised by the Board.

Enquiries:

Dr Michael Thurn
Chief Executive Officer
investorenquiries@pharmaust.com

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

About Motor Neurone Disease:

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 a treatment that prolongs life on average by 2-3 months reaching annual peak sales of ~US$1Bn.

The disease is progressive, meaning the symptoms get worse over time. MND has no cure and no effective treatment to reverse its progression. Independent studies have shown that one-third of patients die within 12 months after the first diagnosis.

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.

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 studies in humans and Phase 2 clinical studies 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 studies.