PharmAust completes Phase 1 MEND Study and files for Orphan Drug Designation

Highlights:

- **All patients have completed the Phase 1 MEND Study of monepantel to treat motor neurone disease**
- **Release of top-line results remains on track for Q1 CY24 data**
- **PharmAust has applied for an Orphan Drug Designation with the US FDA**
- **All patients remain able to swallow and breathe unassisted**
- **All patients are continuing to receive MPL under a compassionate use program**
- **All patients have requested to move to the 12 month open-label trial**

1 December 2023 – Perth, Australia: PharmAust Limited (ASX: PAA) (Company), a clinical-stage biotechnology company, is pleased to announce that all patients have completed its Phase 1 MEND Study of monepantel (MPL) for motor neurone disease (MND/ALS). The release of top-line results remains on track for Q1 CY24.

PharmAust Chief Executive Officer Dr Michael Thurn commented:
"Excitement is growing knowing that we are only months away from releasing top-line data.

We have several patients who have now received treatment with monepantel for 13 months or more, with the median treatment duration being 10.6 months for the 12 patients who participated in the study.

Every month a patient continues on treatment brings enormous satisfaction and excitement around the potential benefit that monepantel may bring to patients, their families and caregivers, and of course the healthcare system as a whole."

About the Phase 1 MEND Study

The Phase 1 MEND Study is an open-label, multicentre study involving 12 patients with MND/ALS to determine the recommended Phase 2 dose based on safety and preliminary efficacy. The study design involves two cohorts of six patients, each 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. The highest dose evaluated was 10 mg/kg.

To date, there have been no deaths or treatment-related serious adverse events. Only three adverse events possibly related to treatment with monepantel have been recorded. There have been no reports of difficulty in swallowing tablets or patients needing assistance with breathing, which are common clinical signs of late-stage disease progression. All patients are continuing treatment with monepantel under a compassionate use program and will be invited to participate in a 12-month open-label extension study due to commence in or around January 2024.

The study is supported by a drug development grant of $881,085 from FightMND, Australia's largest independent not-for-profit organisation for MND research. The final instalment payment of $150,142.80 (plus GST) was received from FightMND in November 2023.

Future Directions
PharmAust has successfully submitted for approval the protocol for the 12-month Open Label Extension (OLE) Study to the Human Research Ethics Committee (HREC). In addition, the Company has applied for an Orphan Drug Designation with the United States (US) Food and Drug Administration (FDA) and remains on track to commence a Phase 2 Study for MND/ALS in H1 CY24.

The FDA has the authority to grant orphan drug designation (ODD) to a drug to prevent, diagnose or treat a rare disease or condition, defined as any disease or condition that affects less than 200,000 persons in the US. An ODD qualifies sponsors for incentives, including tax credits for qualified clinical trials, exemption from user fees, and the potential for seven years of market exclusivity after approval.

The Board authorises this announcement.

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 the standard of care treatment, Riluzole only prolonging life on average by 2-3 months.

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.