Ethics Committee Approval for Open-Label MND Extension Study

Highlights:

- **PharmAust receives approval for Open-Label Extension study of monepantel**
- **All Phase 1 MEND Study patients have expressed interest in continuing treatment and participating in the study**
- **The study is expected to commence in February 2024**
- **The Phase 1 MEND Study and compassionate-use program have seen patients on monepantel for more than 15 months**

30 January 2024 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAOA) ("PharmAust" or "the Company"), a clinical-stage biotechnology company, is pleased to announce it has received approval from Monash Health Human Research Ethics Committee (HREC) to commence an Open-Label Extension (OLE) study of monepantel (MPL) in patients with Motor Neurone Disease (MND)/Amyotrophic Lateral Sclerosis (ALS) at Calvary Health Care Bethlehem, Melbourne.

The OLE study is a multicentre, 12-month study designed to allow the 12 patients who participated in the Phase 1 MEND study to continue to receive treatment with MPL. Patients will receive a daily dose of 10 mg/kg body weight of MPL for 12 months. The study is expected to commence at Calvary Health Care Bethlehem led by Associate Professor Susan Mathers, in February 2024, with patients at Macquarie University, led by Professor Dominic Rowe, to follow soon after.

The OLE study will further test the hypotheses that MPL administration to individuals living with MND/ALS will safely reduce disease-associated protein accumulation in motor neurons and provide therapeutic benefits.

The study's primary objective is to assess the long-term safety and tolerability of MPL. The secondary objective is to assess the biomarkers (serum neurofilament/light chain and Urinary $p75^{ECD}$ levels) and efficacy endpoints (disease severity, cognitive, respiratory, and quality of life assessments using ALS Functional Rating Scale-Revised, Edinburgh Cognitive & Behavioural ALS Screen, Slow Vital Capacity, and ALSSQOL-R respectively).

PharmAust Chief Executive Officer Dr Michael Thurn commented:
"We are delighted to receive ethics approval for this Open-Label Extension study of monepantel in MND/ALS patients. It is very positive that all 12 patients who completed the Phase 1 MEND study are pleased and available to continue treatment with monepantel for a further 12 months. This study allows us to capture safety and efficacy data within a quality framework suitable for regulatory submission.

We are thrilled to work again with experienced investigators Associate Professor Susan Mathers and Professor Dominic Rowe. Together, we hope our efforts bring about a much-needed new therapy for MND/ALS."

With the receipt of HREC approval, the Company has now completed the registration of the OLE study on the ClinicalTrials.gov online registry. Please follow the link below to learn more about the study:
https://clinicaltrials.gov/study/NCT06177431

Compassionate-Use Program
All 12 patients have continued treatment with MPL in order to be eligible for the OLE study through a compassionate-use program administered by their treating physician. In this case either Associate Professor Susan Mathers or Professor Dominic Rowe. The first group of 6 patients that were enrolled in the Phase 1 MEND Study have all received treatment with MPL for more than 15 months now.

The Board authorises this announcement.

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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 (ASX Code: PAA). PAA is a clinical-stage biotechnology company developing therapeutics for human and animal health applications. The company is focused on repurposing monepantel (MPL) for human neurodegenerative diseases and treating cancer in dogs.

MPL is a potent and safe inhibitor of the mTOR pathway. This pathway plays a central role in cell growth and proliferation of cancer cells and degenerating neurons. The mTOR pathway regulates the cellular “cleaning process”, where toxic protein is broken down into macromolecules to be reused. This autophagic process is disrupted in most neurodegenerative diseases, including motor neurone disease (MND/ALS).

PAA’s lead MPL program is for the treatment of MND/ALS, a rare, incurable disease. The company is currently completing a Phase 1 study in patients with MND/ALS. Top-line results are expected to be announced in Q1 CY2024. PAA anticipates starting a Phase 2 study in H1 2024 that could lead to accelerated approval with the US Food and Drug Administration in 2026. PAA is preparing to start a pivotal field trial in dogs with B-Cell Lymphoma to enable product registration in the US in 2025. PAA has previously successfully completed a Phase 1 oncology clinical study of monepantel in humans and pilot studies in canine cancer.