

Final Ethics Committee Approval for Open-Label MND Extension Study

Highlights:

- **Macquarie University has received ethics approval allowing the final 3 patients to be enrolled in the Open-Label Extension study of monepantel**
- **The study commenced at Calvary Health Care Bethlehem in February 2024 and 9 of the 12 eligible patients have enrolled on the study to date**
- **The Phase 1 MEND study, compassionate-use program, and OLE study have now seen patients treated continuously with monepantel for up to 19 months**

10 April 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 Macquarie University 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 Macquarie University, Sydney, allowing the remaining 3 patients to be enrolled.

The multicentre 12-month OLE study, using a daily dose of 10mg/kg body weight of MPL, is designed to collect ongoing data from the 12 patients who completed the Phase 1 MEND study.

The OLE study commenced at Calvary Health Care Bethlehem in February 2024, where Associate Professor Susan Mathers enrolled 9 patients. Professor Dominic Rowe, from Macquarie University, expects to complete enrolment of the final 3 patients this month.

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).

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.

Patients have now been treated continuously for up to 19 months without safety concerns. For these 12 patients the average time since diagnosis is now 29.9 months (minimum 17.3 months and maximum 50.3 months). Population-based prospective registries report 1 year mortality rates after diagnosis ranging from 22% to 34%¹.

PharmAust Chief Executive Officer Dr Michael Thurn commented:

“We are delighted to receive ethics approval from Macquarie University HREC and excited to now be able to offer all Phase 1 MEND patients the opportunity to enrol in the Open-Label Extension study. It's incredibly rewarding to know that 9 patients have already been enrolled at Calvary Health Care Bethlehem and the remaining 3 patients can now continue receiving benefit from treatment with monepantel.

We are very thankful to the patients and our investigators, Associate Professor Susan Mathers and Professor Dominic Rowe, for their ongoing involvement as we continue working towards a much-needed new therapy for MND/ALS.”

Please follow the ClinicalTrials.gov online registry link below to learn more about the study:
<https://clinicaltrials.gov/study/NCT06177431>

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 PharmaAust Limited:

PharmaAust 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.

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 recently announced positive top-line results for its Phase 1 study in patients with MND/ALS. PAA anticipates commencing enrolment in the pivotal registration adaptive Phase 2/3 clinical study in H2 CY 2024 that could lead to accelerated approval with the US Food and Drug Administration in 2026.

The Neurodegenerative Disease Market size is estimated at USD 55.12 billion in 2024, and is expected to reach USD 77.82 billion by 2029, growing at a CAGR of 7.14% during the forecast period (2024-2029)².

¹ Wolf, J., Safer, A., Wöhrle, J.C. et al. Factors predicting one-year mortality in amyotrophic lateral sclerosis patients - data from a population-based registry. BMC Neurol 14, 197 (2014). <https://doi.org/10.1186/s12883-014-0197-9>

² <https://www.mordorintelligence.com/industry-reports/neurodegenerative-disease-market>

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