PharmAust receives Orphan Drug Designation for monepantel

17 May 2024 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAOA) ("PharmAust" or "the Company"), is pleased to announce it has received Orphan Drug Designation (ODD) from the United States (US) Food and Drug Administration (FDA) for monepantel and its treatment of Motor Neurone Disease (MND) / Amyotrophic Lateral Sclerosis (ALS).

The FDA grants ODD status to assist and encourage companies to develop safe and effective treatments for rare diseases and disorders (impacting less than 200,000 persons in the US). Under the US Orphan Drug Act, Orphan Drug status provides incentives, including tax credits, grants, waiver of some administrative fees for clinical trials, and seven years of market exclusivity following drug approval.

PharmAust's application was based on preclinical mechanistic data that demonstrates MPL can induce autophagy in diseased cells, and consideration of the pathology of the disease. PharmAust initially submitted the request for ODD to the FDA's Office of Orphan Products Development early in November 2023. In January 2024, the FDA responded, requesting further supporting data to establish the drug's potential for effectiveness in MND/ALS. In March, PharmAust submitted the positive clinical data from its Phase 1 MEND study involving 12 patients with MND/ALS.

The positive top-line data from the Phase 1 MEND Study was based on daily administration of MPL over a 7 to 12-month period was well-tolerated and did not result in any dose-limiting toxicities or serious adverse effects. Daily administration of MPL resulted in a clinically meaningful therapeutic effect as evidencd by a small and non-significant numerical reduction in ALS Functional Rating Scale-Revised (ALSFRS-R) scores from baseline to end of treatment. Quality of life (ALSSQOL-R Quality of Life Questionnaire) and cognitive and behavioural (Edinburgh Cognitive and Behavioural ALS Screen) function were also not significantly impacted, and there was no change in respiratory function (Slow Vital Capacity). The effects of treatment with MPL on biomarkers demonstrated a large reduction in cerebrospinal fluid (CSF) NfL, which supported a meaningful clinical effect.

John Clark, PharmAust's Managing Director, commented: "This is an outstanding milestone for PharmAust and monepantel, providing an even stronger pathway forward for the drug, particularly in light of recent failures of other MND/ALS treatments. We are now increasingly optimistic as we progress to our pivotal registration adaptive Phase 2/3 study which will commence in H2 CY2024."

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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.

MPL is a potent and safe inhibitor of the mTOR pathway. This pathway plays a central role in the 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 neuron 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 its 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).[1]

[1] https://www.mordorintelligence.com/industry-reports/neurodegenerative-disease-market

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