FDA Provides Clear Feedback for ODD Approval

Highlights:

- **The FDA has requested clinical trial data to support the granting of Orphan Drug Designation for the treatment of MND/ALS**
- **PharmAust is due to release its Phase 1 MEND Study clinical data this quarter and will submit an amendment to the FDA following this release**
- **PharmAust will be allowed to submit an amendment rather than reapplying with an entirely new application**

29 January 2024 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAOA) ("PharmAust" or "the Company"), a clinical-stage biotechnology company, is updating its shareholders that the United States (US) Food and Drug Administration (FDA) Office of Orphan Products Development has requested additional data to support the monepantel (MPL) Orphan Drug Designation (ODD) application for the treatment of Motor Neurone Disease (MND) / Amyotrophic Lateral Sclerosis (ALS). The request has been made due to the absence of preclinical or clinical data to establish the potential for the drug to be effective in MND/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 submitted the request for ODD early in November 2023 to the FDA's Office of Orphan Products Development. The application was based on preclinical mechanistic data that demonstrates MPL can induce autophagy in diseased cells, and consideration of the pathology of the disease. The FDA's reply clearly states that it could not grant the ODD at this stage and requests the provision of preclinical data in a relevant animal model or clinical data from human trials, to establish the potential for the drug to be effective in MND/ALS.

The FDA has provided PharmAust with a pathway to address the absence of clinical data by submitting an amendment to the ODD request rather than reapplying with an entirely new application. PharmAust's Phase 1 MEND Study top-line results are on track to be released before the end of this current quarter, and the Company plans to submit these results to the FDA to support the ODD.

The Phase 1 MEND Study involved 12 patients with MND/ALS and was completed in December 2023. All patients have since elected to continue to receive MPL under a compassionate-use program and are eligible to enrol in a 12-month Open-Label Extension Study due to commence in February 2024. Patients have been treated with MPL for an average of 13 months, and it continues to be well-tolerated by these patients.

PharmAust Chief Executive Officer Dr Michael Thurn commented:
"The Company is encouraged by the clear and concise feedback from the FDA on what is required for the ODD to be granted. We look forward to releasing our Phase 1 MEND Study clinical data in the coming weeks and responding to the FDA to complete the ODD application.

We appreciate the FDA's feedback on our development programs, which allows us to refine our strategies to maximise our opportunities for blockbuster success. Over the coming weeks, we will continue our communication strategy with the FDA about the ODD and the pre-IND meeting for our adaptive Phase 2/3 study."

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.