FDA Grants PharmAust Pre-IND Meeting

Highlights:

- **US FDA grants PharmAust a Pre-IND meeting for monepantel for the treatment of MND/ALS**
- **The meeting provides PharmAust with the opportunity to receive feedback from the FDA on the proposed ongoing development program to receive accelerated approval**
- **FDA has committed to provide written responses by 13 February 2024**
- **PharmAust remains on-track for near-term milestones, including opening an IND application and initiating the adaptive Phase 2/3 clinical study, in Q2 2024**

2 January 2024 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAOA) ("PharmAust" or "the Company"), a clinical-stage biotechnology company, is pleased to announce the United States (US) Food and Drug Administration (FDA) has granted PharmAust a Pre-Investigational New Drug (Pre-IND) meeting for monepantel for the treatment of motor neurone disease/amyotrophic lateral sclerosis (MND/ALS). The request was submitted on 15 December 2023, and the FDA has committed to provide written responses by 13 February 2024.

The request for a pre-IND meeting formally initiates communications with the FDA regarding developing monepantel to treat MND/ALS. The pre-IND meeting aims to confirm the details and acceptability of PharmAust's proposed ongoing development program, including the requirements for non-clinical and clinical pharmacology, clinical chemistry, and manufacturing controls. Reviewers from the FDA's Office of Neuroscience - Division of Neurology I, across multiple disciplines including Medical, Pharmacology/Toxicology, Statistical, Clinical Pharmacology/Biopharmaceutics, and Chemists, will provide feedback on PharmAust's proposed development program for monepantel.

Importantly, and given the stage of development of monepantel for the treatment of MND/ALS, it provides PharmAust with an opportunity to seek feedback from the FDA on the design of its planned Phase 2/3 adaptive clinical study and gain insights into the FDA's requirements for monepantel to be potentially granted accelerated approval. With these considerations in hand, PharmAust will be able to proceed confidently with its full IND application filing in Q2 2024.

PharmAust Chief Executive Officer Dr Michael Thurn commented:
"We are looking forward to receiving feedback from the FDA. It's remarkable to think that PharmAust is potentially one clinical study away from receiving accelerated approval and providing MND/ALS patients with a much-needed new therapy for a condition that is invariably fatal. Receiving feedback from the FDA in February positions us well to achieve our near-term milestone of opening an IND in Q2 2024 with the goal of initiating the planned adaptive Phase 2/3 clinical study in patients shortly after that."

PharmAust's planned Phase 2/3 study is a multicentre, randomised, placebo-controlled, adaptive clinical study evaluating the safety and efficacy of monepantel in patients with MND/ALS over 48 weeks. The primary aim will be to assess the efficacy of monepantel, as compared to placebo, on the progression of MND/ALS. This will be assessed as a change from baseline disease severity measured by the ALS Functional Rating Scale-Revised (ALSFRS-R) total score and survival. As this is an adaptive study design, an interim analysis will be performed at Week 24 by a team of unblinded statisticians for the potential to stop the study early for either success or futility.

PharmAust recently announced that FightMND had notified that its Letter of Intent was successful and invited PharmAust to submit a complete grant application by the 24 March 2024 to help offset the planned adaptive clinical study costs. Successful recipients will be notified in July 2024.

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 (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.