PharmAust Partners with Berry Consultants for Phase 2/3 MND Study

Highlights:

- **PharmAust has partnered with leading MND/ALS clinical study design and statistical analysis specialists Berry Consultants**
- **US-based Berry Consultants has a strong track-record of innovative clinical trial design and experience with the FDA**
- **Berry Consultants participated in the advisory committee meeting for the recent US FDA accelerated approval of Amylyx Pharmaceutical's Relyvrio® for MND/ALS and have also been integral to the Healey ALS Platform Trial**
- **Berry Consultants will oversee the design of PharmAust's adaptive Phase 2/3 study to best position monepantel for FDA approval for the treatment of MND/ALS**

16 January 2024 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAOA) ("PharmAust" or "the Company"), a clinical-stage biotechnology company, is pleased to announce that it has partnered with leading clinical study design specialist Berry Consultants to design and analyse the planned adaptive Phase 2/3 clinical study for monepantel in patients with Motor Neurone Disease (MND)/ Amyotrophic Lateral Sclerosis (ALS).

PharmAust Chief Executive Officer Dr Michael Thurn commented:
"We are delighted to be working with Berry Consultants as their reputation and experience in designing and conducting the statistical analysis of MND/ALS studies is unmatched. Berry Consultants has partnered with the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital. The Center is currently running the largest ALS study ever undertaken, the Healey ALS Platform Trial, which is focused on accelerating the development of promising new drugs for patients with ALS. Berry Consultants was integral in the concept, design, and continual evolution of that study in addition to numerous other MND/ALS programs."

The lead statistician for PharmAust's Phase 2/3 study will be Berry Consultants Director & Senior Statistical Scientist Dr Melanie Quintana, an expert in innovative clinical trial design of rare neurodegenerative diseases.

PharmAust recently announced that the United States (US) Food and Drug Administration (FDA) granted it a Pre-Investigational New Drug (Pre-IND) meeting. The major focus of the meeting will be to receive feedback and advice on the design of the adaptive Phase 2/3 study. Berry Consultants has been instrumental in the design of the adaptive Phase 2/3 study to best position monepantel for FDA approval for the treatment of MND/ALS.

Berry Consultants' Dr Melanie Quintana commented:
"We are excited to partner with PharmAust in the design of its Phase 2/3 study in MND/ALS. ALS is a devastating disease in which there is a clear unmet medical need for treatments that slow and ultimately stop the progression of the disease. Our goal in the design of this study is to rigorously investigate as efficiently as possible whether or not monepantel is effective in slowing ALS disease progression through the use of adaptive design."

About Berry Consultants
Berry Consultants is a statistical consulting company based in Austin, Texas USA, specializing in innovative clinical trial design, analysis, execution, and software solutions for the pharmaceutical and medical device industry. Berry Consultants employs world-renowned experts in Bayesian statistics and strives to set the standard for adaptive clinical trial design and analysis across all medical disciplines. www.berryconsultants.com

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.