PharmAust receives ethics approval for Phase I trial of monepantel in motor neurone disease

- 12 trial subjects to test safety and some efficacy measures
- Special tablets to be developed and manufactured for the trial
- Trial to commence in CYQ4 2021
- Trial funded by FightMND Australia ($881,085)
- Protocol accepted and accessible on US NIH National Library of Medicine ClincialTrials.gov website

2 June 2021 – Perth, Australia: PharmAust Limited (ASX:PAA), a clinical-stage biotechnology company, will proceed with a new Phase I clinical trial in humans to assess monepantel (MPL) in patients with amyotrophic lateral sclerosis/motor neurone disease (ALS/MND).

This trial will be funded and conducted in collaboration with FightMND, Calvary HealthCare Bethlehem (Melbourne) and Macquarie University (Sydney).

ALS/MND is a group of diseases that affect nerve cells controlling our vital everyday functions including movement and breathing. ALS/MND is rare and invariably fatal (~140,000 new patients worldwide per annum) and imposes a high burden on patients, their families and carers, and carries substantial socio-economic costs. Two Australians will be diagnosed with MND every day with a life-time risk of developing MND of 1 in 300. The average life expectancy with MND is just 27 months. There is a great need for better treatments to prolong life and improve therapy.

PharmAust has already shown in its preclinical programs that MPL has the potential to activate molecular pathways relevant to the treatment of MND. If effective, MPL would reduce the rate of degeneration and loss of motor neurons in the anterior horns and motor nuclei of the brainstem. Furthermore, there are a number of surrogate clinical endpoints that will also be determined during the trial. For the purpose, PharmAust is developing and manufacturing a bespoke monepantel tablet for the trial.

Monash Health Human Research Ethics Committee (HREC) has approved the monepantel (MPL) clinical trial protocol.

The study is a multi-centre open label trial entitled *A Phase I Tolerability, Safety, Pharmacokinetics and Preliminary Efficacy Study of Oral Monepantel in Individuals with Motor Neurone Disease*. It is designed to first test safety in 12 individuals living with ALS/MND according to a conventional dose escalation design, with each level of the dose escalation lasting 28 days. Measures of efficacy are included in the trial design so that where appropriate, this trial can be extended into a Phase II setting. Details of the trial can be found at clinicaltrials.gov using the Identifier code: NCT04894240.

PharmAust expects that in due course MPL will receive orphan drug designation by the FDA for the indication of motor neurone disease. Such designations come with a number of financial and supportive benefits. The Orphan Drug Act provides for granting special status to a drug or biological product to treat a rare disease or condition upon request of a sponsor.

Acceptance is conditional upon a minor update to the Ethics committee providing the location of laboratories where biospecimens collected during the trial will be forwarded for analysis. The consortium is finalising negotiations with several laboratories possessing the required capabilities. Trial commencement is anticipated for CYQ4 2021.

FightMND has committed more than $48m to research to help find a cure for motor neurone disease since former AFL player and coach Neale Daniher founded the charity in 2014. Big Freeze 7 will see the famous ice slide return to the MCG when Melbourne takes on Collingwood on the Queen's Birthday June 14 holiday. Several big names from the football and entertainment worlds are already lined up to take the icy plunge.

As announced on 21 September 2020, FightMND awarded PharmAust and the Principal Investigators Dr Susan Mathers of Calvary HealthCare Bethlehem and Professor Dominic Rowe of Macquarie University $881,085 to undertake this trial. PharmAust and Dr Mathers and Professor Rowe are grateful for the support from FightMND and look forward to providing trial outcomes as they become known.

PharmAust's Chief Scientific Officer, Dr Richard Mollard stated, "PharmAust is privileged and delighted to be working with Dr Mathers, Professor Rowe, FightMND, Monash Health and Macquarie University with the common aim of finding positive outcomes for people living with ALS/MND. PharmAust is especially thankful to FightMND and all its supporters who make this type of work possible."

Dr Susan Mathers stated, "Along with patients and colleagues, I am looking forward to commencing this clinical trial. The Australian community has committed to the fight against MND and I thank them for their generous and spirited support for research and for the well-being of people living with this disease."

Dr Bec Sheean, Research Director at FightMND commented, "FightMND is pleased to be supporting this trial for MND patients in Australia. We look forward to the results and outcomes of this Phase I trial, in the hope that promising results will support the progression of monepantel to a Phase II trial and beyond."

Approval for the trial is given in accordance with the research conforming to the Australian National Health and Medical Research Council Act 1992 and the Australian National Statement on Ethical Conduct in Human Research (2018). The Monash Health HREC has ethically approved this research according to the Memorandum of Understanding between the Victorian Department of Health and Human Services and the participating organisations conducting the research.

This announcement is authorised by the Board

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About PharmAust (PAA):

PharmAust Limited is listed on the Australian Securities Exchange (code: PAA) and the Frankfurt Stock Exchange (code: ECQ). PAA is a clinical-stage company developing therapeutics for both humans and animals. The company specialises in repurposing marketed drugs lowering the risks and costs of development. These efforts are supported by PAA's subsidiary, Epichem, a highly successful contract medicinal chemistry company that generated $3.5 million in revenue in FY 2020.

PAA's lead drug candidate is monepantel (MPL), a novel, potent and safe inhibitor of the mTOR pathway – a pathway having key influences in cancer growth, neurodegenerative diseases and viral infections. MPL has been evaluated in Phase 1 clinical trials in humans and Phase 2 clinical trials in dogs. MPL treatment was well-tolerated in humans, demonstrating preliminary evidence of anticancer activity. MPL demonstrated objective anticancer activity in dogs. PAA is uniquely positioned to commercialise MPL for treatment of human and veterinary cancers as well as neurodegenerative and antiviral disease as it advances a reformulated version of this drug through Phase 1 and 2 clinical trials.

About FightMND:

Founded in 2014, FightMND was established in Australia with the purpose of finding effective treatments and ultimately a cure for Motor Neuron Disease (MND), also referred to as ALS or Lou Gehrig's Disease. FightMND, with its vision of a world without MND, is the largest independent funder of MND research in Australia. What FightMND has done since 2014, is be the voice and the guiding star for Australians who want to fight "The Beast". Integral to this vision is the determination to help facilitate the translation of the growing body of new knowledge about the disease into a cure for MND patients in Australia and abroad.

For more information about FightMND, visit the website at https://fightmnd.org.au.