



PharmAust Awarded Grant for Monepantel Phase I trial in Motor Neurone Disease

- PharmAust receives A\$881,085 from FightMND for Phase I trial in humans with Motor Neurone Disease (MND).
- Trial to recruit people with MND at 2 sites:
 - Calvary Health Care Bethlehem, Melbourne. Coordinating Principal Investigator, Dr Susan Mathers
 - Macquarie University, Sydney, Principal Investigator, Professor Dominic Rowe.
- FightMND funds for:
 - dedicated monepantel tablet manufacturing program
 - trial participant assessment and monitoring during the trial
 - Independent clinical research organisation to assist trial management
- Trial to begin 2021.

21 September 2020 – Perth, Australia: PharmAust Ltd (ASX:PAA), a clinical-stage oncology company, has received funding of A\$881,085 for a Phase I trial examining the effects of monepantel (MPL) in Motor Neurone Disease (MND), otherwise known as Lou Gehrig Syndrome or Amyotrophic Lateral Sclerosis (ALS).

These funds have been granted by FightMND, the largest independent funder of MND research in Australia. The trial will be overseen by Dr Susan Mathers of Calvary Health Care, Bethlehem, Melbourne, and will include a second trial site headed by Professor Dominic Rowe of the Centre for Motor Neurone Disease Research Faculty of Medicine and Health Research at Macquarie University in Sydney.

ALS/MND is a rare and invariably fatal neurological disease. ALS/MND has a terribly high burden on patients, family and carers, and carries a great socio-economic cost. Current best treatment options attempt to control disease progression and manage symptoms while offering no cure. These are expensive. Better treatments are sorely needed.

As an mTOR inhibitor, MPL induces autophagy, which is a mechanism relevant to the clearing of misprocessed and excessive intracellular protein in neural cells associated with the cause of ALS/MND. It is therefore hypothesised that MPL will benefit the health of patients' neural cells by regulating problems associated with these proteins, thereby reducing ALS/MND-associated symptoms. Of interest, it has been recently demonstrated that mTOR inhibition through autophagy synergises therapeutically with Riluzole, the current ALS/MND standard-of-care. This means that success in this trial may provide greater options to patients through the use of either stand-alone or combinational therapeutics.

Preparations for the trial have already commenced and while remaining subject to approval from the Institutional Human Research Ethics Committees, Phase I trial recruitment will commence as soon as possible in 2021. The funding agreement provides that PharmAust shall own all intellectual property generated from the study.

PharmAust's Chief Scientific Officer Dr Richard Mollard stated, "PharmAust is delighted to be a successful applicant for the FightMND Research Fund. PharmAust acknowledges FightMND and its donors, in particular the patients, their families and friends and their support network. PharmAust is looking forward to assisting Dr Mathers and Dr Rowe in this trial."

This announcement is authorised by the Board

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About PharmAust (PAA):

PAA is a clinical-stage company developing targeted cancer therapeutics for humans and animals. The company specialises in repurposing marketed drugs lowering the risks and costs of development

PAA's lead drug candidate is monepantel (MPL), a novel, potent and safe inhibitor of the mTOR pathway – a key driver of cancer. MPL has been evaluated in Phase 1 clinical trials in humans and dogs; was well tolerated and produced a significant reduction in key prognostic biomarkers. PAA is positioned to commercialise MPL for treatment of human and veterinary cancers as it advances the drug in Phase 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>.