# Monepantel Selected for Entry into HEALEY ALS PLATFORM TRIAL

**Highlights:**

- **PharmAust's lead investigational drug, monepantel has been accepted into the prestigious HEALEY ALS Platform Trial in the US**
- **Selection provides independent validation by one of the global leaders in ALS on monepantel's potential as an ALS treatment**
- **FDA-approved HEALEY ALS Platform Trial is a large-scale collaboration across multiple clinical trial sites, industry partners and researchers to evaluate multiple drug candidates for the treatment of ALS**
- **Provides additional exposure within the world's ALS research community**

**15 July 2024 – Melbourne, Australia:** PharmAust Limited (ASX:PAA) (**PharmAust** or the **Company**) is pleased to announce that monepantel has been selected for inclusion in the HEALEY ALS Platform Trial under a Clinical Research Support Agreement (CRSA) with Massachusetts General Hospital (MGU). This marks an important step as PharmAust progresses monepantel (MPL) towards seeking United States (US) Food and Drug Administration (FDA) approval for the treatment of amyotrophic lateral sclerosis (ALS) and provides independent validation of MPL's potential as an ALS treatment.

The HEALEY ALS Platform Trial is an innovative Phase 2/3 platform trial structure that allows for multiple investigational treatments to be tested simultaneously using a shared master protocol. The platform trial model, successfully utilized in oncology, aims to expedite the study of multiple therapies, allowing investigators to test more potential therapies, increase patient access, reduce costs, and shorten development timelines. With over 70 trial sites across the United States, the platform aims to enrol 160-240 participants per regimen, offering an optimised 3:1 active drug to placebo ratio. Drug candidates that enter the platform trial are chosen by a group of expert ALS scientists and members of the Healey & AMG Center Science Advisory Committee.

**Merit Cudkowicz, MD, director of the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital, Chair of the Department of Neurology, and Principal Investigator of the HEALEY ALS Platform Trial commented:**
"I look forward to working with PharmAust and studying monepantel in an accelerated format through the HEALEY ALS Platform Trial. The design team will work closely with PharmAust on their regimen specific protocol in readiness for submission to the FDA."

Under the CRSA, the HEALEY ALS Platform Trial design team will work with PharmAust to develop a regimen specific protocol with information specific to monepantel. Study biostatisticians and PharmAust will adapt the regimen-specific Statistical Analysis Plans, as necessary, to incorporate considerations specific to the monepantel study regimen.

**PharmAust Managing Director Dr Michael Thurn commented**:
"We are excited to partner with the HEALEY ALS Platform Trial, a prestigious and well-recognised initiative in the field of ALS research, providing invaluable exposure within the ALS research community. This partnership marks a significant step forward in our efforts to develop monepantel as a viable treatment for ALS. The collaboration with leading ALS experts and the streamlined regulatory support will accelerate our progress towards delivering a much-needed therapy for patients with ALS.

"While we had been exploring the potential to conduct the adaptive Phase 2/3 STRIKE study globally, including at sites in Australia, the opportunity to be part of the HEALEY ALS Platform Trial via their US

network of 72 clinical sites is substantial. We remain committed to supporting the ALS community in Australia and ultimately the HEALEY ALS Platform Trial will provide us with the most efficient and safest way to bring monepantel to all patients. This collaboration with the HEALEY ALS Platform Trial, with its streamlined regulatory support and engagement with leading ALS experts, is a critical advancement in our mission to offer a viable treatment for ALS."

For more information about the HEALEY ALS Platform Trial, please visit their website.

PharmAust remains dedicated to advancing novel treatments for neurological diseases, with a strong focus on maximising the therapeutic potential of MPL in ALS and other challenging conditions.

This announcement is authorised for release by the Board of Directors of PharmAust Limited.

**For further information, please contact:**

**Dr Michael Thurn**
Managing Director
investorenquiries@pharmaust.com
P +61 (3) 9692 7222
W www.pharmaust.com

**Media:**
Catherine Strong
Morrow Sodali
c.strong@morrowsodali.com
P 0406 759 268

**About the Sean M. Healey & AMG Center**

The Sean M. Healey & AMG Center at Massachusetts General Hospital, in collaboration with the Northeast ALS Consortium (NEALS), has launched the first ever platform trial for ALS. This project aims to greatly accelerate the timelines towards effective ALS treatments and to provide greater trial access for patients affected by this devastating disease.

The HEALEY ALS Platform Trial's investigational new drug application (IND) was approved by the FDA in January 2020 and is a collaborative effort with the initial goal of 54 clinical trial sites prepared to enroll patients this year across the U.S. to provide greater access to patients wishing to participate.

**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 neurodegenerative diseases. The company is focused on repurposing monepantel (MPL) for amyotrophic lateral sclerosis (ALS). ALS is the most common form of motor neurone disease (MND) and affects both upper and lower motor neurons.

MPL is a potent and safe inhibitor of the mTOR pathway. This pathway plays a central role in the growth and proliferation of cancer cells and degenerating neurons. The mTOR pathway regulates the cellular “cleaning process”, where toxic proteins are broken down into macromolecules to be reused. This autophagic process is disrupted in most neurodegenerative diseases, including ALS.

The company recently announced positive top-line results for its Phase 1 MEND study in participants with ALS. MPL has been selected for inclusion in the HEALEY ALS Platform Trial and anticipates commencing enrolment in Q4 CY 2024. This single pivotal study could potentially lead to accelerated approval with the US Food and Drug Administration for monepantel for the treatment of ALS in 2026.

In 2024, the Neurodegenerative Disease Market size is estimated to be worth USD 55.12 billion, with a forecast growth (CAGR) of 7.14% the market size is expected to reach USD 77.82 billion by 2029.[1]

[1] https://www.mordorintelligence.com/industry-reports/neurodegenerative-disease-market

**PharmAust Investor Hub:**

We encourage you to utilise our Investor Hub for any enquiries regarding this announcement or other aspects concerning PharmAust. This platform offers an opportunity to submit questions, share comments, and view video summaries of key announcements.

Access the investor hub by scanning the QR code or visiting: https://investorhub.pharmaust.com/