

## Berry Consultants Presents PharmAust's Phase 1 Study Data at Major International ALS Conference

### Highlights:

- Dr Philip White and Dr Melanie Quintana of Berry Consultants presented data from PharmAust's successful Phase 1 MEND study at the annual meeting of the European Network to Cure ALS (ENCALS)
- The data highlighted the statistically significant survival benefit for patients treated with monepantel compared to untreated matched-controls from the PRO-ACT database
- Representatives from PharmAust will be in attendance to hold a variety of business meetings
- [Click here to view the poster being presented at the ENCALs meeting](#)
- [Click here to watch a video of Dr Philip White presenting the poster](#)

**18 June 2024 – Melbourne, Australia:** PharmAust Limited (ASX: PAA & PAAOA) ("PharmAust" or "the Company"), a clinical-stage biotechnology company, is pleased to announce that Berry Consultants presented data from PharmAust's successful Phase 1 MEND study at the annual meeting of the European Network to Cure ALS (ENCALS), being held in Stockholm, Sweden from 17-19 June.

The meeting is the largest and most significant motor neurone disease/amyotrophic lateral sclerosis (MND/ALS) conference in Europe, attended by physicians, key opinion leaders, pharmaceutical and biotechnology representatives, academics and patient advocacy groups.

The poster presentation by Dr Philip White and Melanie Quintana highlighted the previously reported (as announced on 14 and 27 February and 5 June) statistical comparisons made between patients treated with monepantel (MPL) in PharmAust's recently completed Phase 1 MEND Study to untreated matched-controls from the Pooled Resource Open-Access ALS Clinical Trials (PRO-ACT) database for patients with MND/ALS.

### The highlights included:

- An average 0.48-point improvement in Revised Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R) score per month for the MPL treatment patients, relative to untreated matched-controls from the PRO-ACT database
- Compared to matched controls from the PRO-ACT database, treatment with MPL results in a significantly ( $\chi^2=9.39$ ,  $p=0.0022$ ) longer survival of patients with MND/ALS
- Treatment with MPL significantly reduces the risk of death by 91% (HR=0.087,  $p=0.0154$ ) compared to PRO-ACT matched controls

PharmAust's Non-Executive Chairman, Sergio Duchini and the Company's Chief Operating Officer, John Clark will be in attendance at ENCALs for a variety of business meetings with industry, global clinical trial service providers and key opinion leaders in MND/ALS.

### **PharmAust Managing Director Dr Michael Thurn commented:**

"To have Dr Philip White and Dr Melanie Quintana present the clinical data from our successful Phase 1 MEND Study at a major international conference such as ENCALs is a major achievement for the Company. We are very grateful to Berry Consultants for independently presenting the data and creating greater awareness about our promising efficacy data to the broader MND/ALS community. We look forward to working with Berry Consultants as we move closer to finalising the design of the pivotal adaptive Phase 2/3 STRIKE study, planned to commence in H2 2024."

### **About the PRO-ACT database**

The PRO-ACT database is the largest publicly available repository of merged ALS clinical study data. Clinical study data were pooled from 16 completed Phase 2/3 ALS/MND clinical studies and one observational study. Over 8 million de-identified longitudinally collected data points from more than 10,000 persons with ALS were standardised across studies and merged to create the PRO-ACT database. This database includes demographics, family histories, and longitudinal clinical and laboratory data.

### **About Berry Consultants**

Berry Consultants is a statistical consulting company based in Austin, Texas USA, specialising 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](http://www.berryconsultants.com)

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

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### **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. PAA is in the planning stages for a registration adaptive Phase 2/3 STRIKE clinical study and anticipates commencing enrolment in H2 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.<sup>1</sup>

<sup>1</sup> <https://www.mordorintelligence.com/industry-reports/neurodegenerative-disease-market>

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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.

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<https://investorhub.pharmaust.com/>

