



## PharmAust Forms World-Class Scientific Advisory Board

### Highlights:

- PharmAust engages globally renowned experts in MND/ALS and rare neurodegenerative diseases to form a Scientific Advisory Board (SAB).
- This SAB will provide expert advice for the development of monepantel to treat MND/ALS and explore other neurodegenerative diseases.
- The SAB will also support the planning stages for PharmAust's adaptive Phase 2/3 clinical study in MND/ALS expected in CY2024.

**22 February 2024 – Perth, Australia:** PharmAust Limited (ASX: PAA & PAAOA) ("PharmAust" or "the Company"), a clinical-stage biotechnology company, is pleased to announce the formation of its Scientific Advisory Board (SAB). The SAB, which includes internationally renowned experts in MND/ALS drug discovery and clinical development, will provide strategic guidance in the development of PharmAust's lead candidate monepantel (MPL) for the treatment of Motor Neurone Disease (MND) / Amyotrophic Lateral Sclerosis (ALS) and pipeline expansion activities in additional neurodegenerative diseases.

### Members of the Scientific Advisory Board include:

**Prof Leonard van den Berg** – Professor of Neurology who holds a Chair in Experimental Neurology of motor neuron diseases at the University Medical Center Utrecht in the Netherlands. He also is Director of the centre's Laboratory for Neuromuscular Disease, Director of the Netherlands ALS Center, Chairman of the Neuromuscular Centre the Netherlands, and Chairman of the European Network to Cure ALS (ENCALS), a network of the European ALS Centres. Prof. van den Berg did a fellowship in neuroimmunology at the Neurological Institute at Columbia University in New York and obtained his PhD degree at UMC Utrecht. He has been Professor of Experimental Neurology since 2005 and leads a research group focused on translational research into ALS and other diseases of motor neurons.

**Dr Sabrina Paganoni** – Co-Director of the Neurological Clinical Research Institute at the Massachusetts General Hospital, Assistant Professor at Harvard Medical School, and physician investigator at the Sean M. Healey and AMG Center for ALS at Mass General. Dr Paganoni's research focuses on clinical trials and therapy development for ALS. She has served as Principal Investigator of several ALS clinical trials. She is the co-Principal Investigator of the HEALEY ALS Platform Trial, the world's first platform trial for ALS. She has published over 100 peer-reviewed manuscripts and received several awards, including the 2021 Top 10 Clinical Research Achievement Award. She co-chairs the Upper Motor Neuron Task Force, the Technology Committee, and the Recruitment/Retention/ Experience Committee at NEALS.

**Dr Melanie Quintana** - Director and Senior Statistical Scientist at Berry Consultants, where she specialises in designing Bayesian adaptive clinical trials across a wide range of therapeutic areas. Her work has included numerous examples in designing platform trials, including the HEALEY ALS Platform Trial and clinical trials in rare and progressive diseases, focusing on developing disease progression models to design better and more powerful clinical trials. Before joining Berry Consultants, Melanie earned her PhD in Statistics from Duke University and pursued a Postdoc in Biostatistics at The University of Southern California.

**Dr Christian Freitag** – brings over 20 years of experience in the pharmaceutical industry with positions in companies including Hoffmann La Roche, Shire, and BTG, where he led global clinical development projects. Dr. Freitag has held the position of Chief Medical Officer at Dynacure and Azafaros, where he was responsible for

medical and regulatory strategy, including clinical development of their lead compound in rare diseases. Dr Freitag was the Medical Monitor on PharmAust's Phase 1 MEND study and oversaw medical and clinical activities.

**Prof Leonard van den Berg commented:**

"I'm very excited to be a part of PharmAust's SAB and look forward to guiding with my fellow SAB members the next phase of clinical development of monepantel for the treatment of MND/ALS. There is a clear need for more effective treatment options for MND/ALS. The survival data produced to date with monepantel is encouraging and compounds that enhance autophagy have the potential to treat a wide range of neurodegenerative diseases".

**PharmAust Chief Executive Officer Dr Michael Thurn commented:**

"We are honoured to work with this prestigious and accomplished group of thought leaders as our Scientific Advisory Board. These innovative individuals have pioneered breakthroughs in MND/ALS, and together they bring a wealth of knowledge and experience to PharmAust. We look forward to collaborating with this group to inform and shape our research and development efforts in advancing our lead monepantel program for treating MND/ALS and the potential expansion of other neurodegenerative indications".

The Board authorises this announcement.

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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 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 an adaptive Phase 2/3 clinical study in H2 CY 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.

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