EMA Pre-Submission Meeting Paves the Way for Orphan Designation Request

Highlights:

- **PharmAust has submitted a request for Orphan Medicinal Product Designation (OMPD) to the EMA for monepantel for the treatment of amyotrophic lateral sclerosis**
- **If granted, OMPD status will provide PharmAust with significant regulatory support and commercial benefits, including ten years post-authorisation market exclusivity in the EU**
- **The OMPD evaluation period has a fixed duration of 90 days**

17 September 2024 – Melbourne, Australia: PharmAust Limited (ASX: PAA & PAAOA) ("PharmAust" or "the Company"), a clinical-stage biotechnology company, is pleased to announce it has completed a Pre-submission meeting with the European Medicines Agency (EMA) and the Committee for Orphan Medicinal Products (COMP) and has now submitted a request for OMPD for monepantel (MPL) for the treatment of amyotrophic lateral sclerosis (ALS). The COMP's decision is expected during December 2024.

The request for OMPD was made following a Pre-submission meeting held in August 2024 with the EMA and COMP. The Pre-submission meeting is encouraged by the EMA and offers companies a valuable opportunity to receive feedback on their draft applications to increase the likelihood of success following a formal OMPD submission. PharmAust already received an Orphan Drug Designation (ODD) from the United States (US) Food and Drug Administration's (FDA's) Office of Orphan Products Development earlier this year for the use of MPL for the treatment of ALS.

PharmAust Managing Director Dr Michael Thurn commented:
"We are encouraged by the feedback received by the EMA on our draft application and invitation to submit a full OPMD application. The evaluation period has a fixed duration of 90 days during which the COMP will consider our application, assessing clinical data alongside other evidence to determine the eligibility of MPL for OMPD. The EU market represents an important market for PharmAust, where the incidence of ALS in European countries are amongst the highest globally, ranging from 1.5 to 3.8 per 100,000 people.[1] In July we received Small Medium Enterprise (SME) status from the EMA and look forward to advising the market of the outcome of the OMPD by the end of the year."

Similar to the FDA's ODD, the OMPD is an EMA initiative to encourage companies to develop safe and effective treatments for rare diseases or neglected severe disorders. OMPDs provide structured regulatory and commercial incentives to companies, including the following:

- **Protocol assistance**
  This allows companies to seek advice on the types of studies needed to demonstrate the medicine's quality, benefits and risks, and information on the significant benefit of the medicine.
- **Central marketing authorisation in the European Union**
  This allows companies to make a single application to the EMA, resulting in a single opinion and a single decision from the European Commission, valid in all EU Member States.
- **Access to conditional approval**
  Potential to receive conditional approval under the centralised procedure.
- **10 years market exclusivity**
  Approved orphan medicines benefit from ten years of protection from market competition with similar medicines with similar indications once they are approved.

- **Reduced fees for regulatory activities**
  This includes reduced fees for protocol assistance, marketing-authorisation applications, inspections before authorisation, applications for changes to marketing authorisations made after approval, and reduced annual fees.
- **SME benefits**
  Companies that have been granted Small Medium Enterprise (SME) status like PharmAust receive further incentives including administrative and procedural assistance from the Agency's SME office and fee reductions.

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 announced positive top-line results for its Phase 1 MEND study in patients with ALS in February this year. MPL was recently 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

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