PharmAust Commences Manufacture of GMP-Grade Monepantel for Human Clinical Trial

- Monepantel (MPL) to be used in a Phase 1/2 human trial in Motor Neurone Disease (MND), as well as a human Phase 1/2 cancer trial
- The MND trial, co-funded with FightMND, is scheduled to commence in October 2021
- Subject to regulatory approvals the cancer trial in humans is scheduled to commence in late CY 2021
- In-house small-scale manufactured MPL demonstrates greater than 2-year shelf-life

23 December 2020 – Perth, Australia: PharmAust Limited (ASX:PAA) is pleased to announce the commencement of production of 10kg of GMP-grade monepantel (MPL) for research and development (R&D) in two Phase 1/2 clinical trials in humans. These are a Phase 1/2 clinical trial examining the effects of MPL in individuals with motor neurone disease (MND), as well as a Phase 1/2 clinical trial examining the effects of MPL-tablets in humans with selected cancers. MPL will be manufactured in collaboration with Syngene International Ltd., an integrated research, development and manufacturing services company.

As previously announced on 29 October 2018, GMP (Good Manufacturing Practice) is a globally recognised standard that requires rigorous, controlled and continually documented processes to provide fully characterised drugs with very high levels of purity for safe and effective use when administered to patients. This level of manufacture meets standards required for clinical trials in Australia, the US and Europe.

India-headquartered Syngene International Ltd will undertake scaled manufacture feasibility studies prior to synthesising the full 10kg of GMP-grade MPL. Synthesis of the GMP grade MPL is expected by June 2021. GMP MPL tablets and MPL tablet stability shelf-life data sufficient to commence the MND clinical trial co-funded with FightMND are expected to be delivered to PharmAust for an October 2021 trial start date.

Following the collection of key data from the MND trial, where drug blood levels following administration of specific tablet doses will be measured, PharmAust will commence a follow-up Phase 1/2 R&D trial in humans with cancer.

Previously PharmAust demonstrated stabilised cancer and blood cancer markers as well as reduced mTOR anticancer markers in humans at known blood drug levels. The trial was stopped early, however, due to the highly unpalatable liquid formulation. Since then PharmAust successfully reformulated MPL into a tablet that was devoid of palatability issues. Following successful preclinical work in rats and dogs comparing the liquid and tablet formulations, PharmAust can now tailor tablet dosage to more meaningfully target drug blood levels known to elicit anticancer activity.

PharmAust's wholly owned subsidiary Epichem Pty Ltd has conducted stability shelf-life tests of MPL over two years using feasibility batches manufactured according to the in-house method developed with Syngene. These initial non-GMP tests suggest that the Syngene MPL has demonstrated purity and stability levels compatible with use in clinical trials. It is therefore anticipated that scaled material for the upcoming clinical R&D trials should demonstrate suitable shelf-life specifications.

PharmAust's Chief Scientific Officer Dr Richard Mollard stated "The commencement of this GMP synthesis program for clinical R&D purposes is a significant next step for PharmAust. Combining the in-house GMP manufacturing method with the in-house tablet formulation provides PharmAust with great operational certainty moving forward into the clinical trials in neurodegenerative diseases and cancer."

Commenting on the collaboration, Jonathan Hunt, Managing Director and Chief Executive Officer, Syngene International Ltd. said "We are delighted to collaborate with PharmAust to manufacture and supply clinical trial batches of monepantel to support Phase 1 and 2 clinical trials in humans. Our cGMP compliant manufacturing facilities in Bengaluru is USFDA approved and has a track record of supporting advanced intermediates and NCEs for molecules in the early and late phase, and commercial supplies."

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About PharmAust Limited:
PAA is a clinical-stage company developing targeted cancer therapeutics for both humans and animals. The company specialises in repurposing marketed drugs lowering the risks and costs of development. PAA is listed on the Australian Securities Exchange (code: PAA) and the Frankfurt Stock Exchange (code: ECQ).

PAA's lead drug candidate is monepantel (MPL), a novel, potent and safe inhibitor of the mTOR pathway – a pathway having key influences in cancer growth and neurodegenerative diseases. MPL has been evaluated in Phase 1 clinical trials in humans and Phase 2 clinical trials in dogs. MPL treatment was well-tolerated in humans, demonstrating preliminary evidence of anticancer activity. MPL demonstrated objective anticancer activity in dogs. PAA is uniquely positioned to commercialise MPL for treatment of human and veterinary cancers as well as neurodegenerative disease as it advances a reformulated version of this drug through Phase 1 and 2 clinical trials.

About Syngene International:
Syngene International Ltd. (BSE: 539268, NSE: SYNGENE, ISIN: INE398R01022), is an integrated research, development and manufacturing services company serving the global pharmaceutical, biotechnology, nutrition, animal health, consumer goods and specialty chemical sectors. Syngene's 4500+ scientists offer both skills and the capacity to deliver great science, robust data management and IP security and quality manufacturing, at speed, to improve time-to-market and lower the cost of innovation. With a combination of dedicated research facilities for Amgen, Baxter, Bristol-Myers Squibb and Herbalife, as well as 1.9 Mn sq ft of specialist discovery, development and manufacturing facilities, Syngene works with biotech companies pursuing leading edge science as well as multinationals including GSK and Merck KGaA. For more details, visit https://syngeneintl.com/

About Epichem:
Epichem is a wholly owned subsidiary of the ASX listed company PharmAust Limited. Located in Technology Park, Western Australia, Epichem has been delivering products and services in synthetic and medicinal chemistry to the global drug discovery and pharmaceutical industries in over 40 countries worldwide for over 17 years. Epichem has newly constructed purpose-built, state-of-the-art laboratories and has world class equipment and expertise in synthetic and medicinal chemistry to support drug discovery projects, and for the cost-effective synthesis of drug analogue libraries and intermediates. It also has a rapidly growing catalogue of pharmaceutical reference standards. Epichem is the winner of the WA Industry Export Award 2019 for International Health, the 2020 Inspiring Story of Celebrating Remarkable Resilience Nomination for WA for the Australian Export and Investment Awards and the 2020 GHP Biotechnology Award winner for Most Innovative Chemistry Service Provider – Australia and Best in Organic Chemistry Solutions 2020. Epichem generated Aus$3.5M in revenues in the 2020 FY. For more information, visit www.epichem.com.au

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.