



ASX RELEASE

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PARADIGM IN-LICENSES TREATMENT FOR RARE JOINT DISEASE

HIGHLIGHTS

- Paradigm has executed an Exclusive License Agreement with the Icahn School of Medicine at Mount Sinai, New York, for the treatment of a rare inherited disease which causes debilitating joint pain and dysfunction
- In a Phase 2 clinical study, subjects with the rare disease have been successfully treated with injectable pentosan polysulfate sodium (iPPS) generating excellent safety and strong efficacy data
- A key unmet medical need in this inherited disease is the lack of treatment of chronic joint pain and dysfunction akin to osteoarthritis
- License includes successful Phase 2a safety and efficacy data which provides highly valuable long-term safety data on iPPS which can be included in US FDA submissions for Paradigm's primary indication (Osteoarthritis/BMEL)
- Paradigm remains on track for the read out of the its Phase 2b OA/BMEL clinical trial in mid-late December 2018.

Melbourne 22 November 2018, Paradigm Biopharmaceuticals Ltd (ASX: PAR) is pleased to announce that it has executed an Exclusive In-License Agreement for the use of iPPS in the treatment of mucopolysaccharidoses (MPS), a group of inherited lysosomal storage disorders.

A key unmet medical need in this class of inherited disease is the lack of treatment of joint pain and dysfunction akin to osteoarthritis.

The Licensing Agreement and accompanying Phase 2a human data is very complementary and synergistic to the Company's OA/BMEL program, as such, it is a logical and valuable addition to Paradigm's product pipeline and IP portfolio at this time.

Like most orphan indications, this iPPS treatment has the potential to be a billion dollar per annum (blockbuster) pharmaceutical product, assuming successful registration.

The Licensing Agreement was executed on attractive commercial terms (single-digit royalty – no payment of equity or dilution to existing shareholders), providing Paradigm with highly valuable Intellectual Property:

- **Patents:** Granted patents in all key regions, USA, Japan, Europe, Australia and New Zealand
- **Clinical Data:** Successful phase 2a clinical trial that demonstrated excellent safety and strong efficacy data. Importantly, the data set includes long term safety of iPPS in very sick and

debilitated patients with chronic joint pain, which would be included in future FDA submissions for Paradigm's primary indications (Osteoarthritis/BMEL)

- **High probability of developing a commercial product:** Based on Paradigm's review of the human clinical data, the product development for the targeted population of people who have inherited the rare disease with joint pain and dysfunction could receive 'fast-track' approval following a pivotal phase 2b clinical trial.
- **Indication will now be included under Paradigm's exclusivity agreement with bene pharmaChem:** This new IP will fall under the same terms of exclusivity and supply that Paradigm currently enjoys with bene pharmaChem.

This newly in-licensed indication is fully funded for the next 12 months and beyond as detailed in the recent capital raising 'Use of Funds'.

The Phase 2b OA/BMEL clinical trial head-line results reads-out in mid to late December 2018, which remains the Company's primary focus.

WHAT IS MPS?

The mucopolysaccharidoses (MPS) are a family of disorders caused by inherited (genetic) defects in the catabolism of sulfated components of connective tissue known as glycosaminoglycans (GAGs). The crude cumulative rate for all types of MPS is around 3.5 in 100 000 live births¹ and generally the patients present in one of three ways:

1. As a dysmorphic syndrome (MPS IH, MPS II, MPS VI) often with early onset middle ear disease, deafness, or upper airways obstruction
2. With learning difficulties, behavioural disturbance and dementia and mild somatic abnormalities (MPS III)
3. As a severe bone dysplasia (MPS IV)

ABOUT THE ICAHN SCHOOL OF MEDICINE & THE MOUNT SINAI HEALTH SYSTEM

The Icahn School of Medicine at Mount Sinai is a New York not-for-profit corporation, which is a leader in medical and scientific training and education, biomedical research and patient care.

The Mount Sinai Health System is New York City's largest integrated health delivery system encompassing seven hospital campuses, a leading medical school, and a vast network of ambulatory practices throughout the greater New York region. Mount Sinai's vision is to produce the safest care, the highest quality, the highest satisfaction, the best access and the best value of any health system in the nation. The System includes approximately 6,600 primary and specialty care physicians; 10 joint-venture ambulatory surgery centers; more than 140 ambulatory practices throughout the five boroughs of New York City, Westchester, Long Island, and Florida; and 31 affiliated community health centers.

ABOUT PARADIGM PHARMACEUTICALS LTD

Paradigm Pharmaceuticals Limited (ASX: PAR) is an ASX-listed biotechnology company focused on repurposing Pentosan Polysulfate Sodium (PPS), an FDA approved drug that has a long track record of safely treating inflammation over 60 years.

The Company is currently undertaking a phase 2b randomised, double blind, placebo controlled multicentre trial, investigating subjects with osteoarthritis and concurrent bone marrow edema

¹ <https://www.sciencedirect.com/topics/agricultural-and-biological-sciences/mucopolysaccharidosis>

lesions. The recruitment of the 110 subjects into the clinical trial commenced in November 2017 and concluded in August 2018. Paradigm will read out the results of this clinical trial in December 2018. There is a global trend for safe and effective non-opioid and non-steroid pain relief for chronic disease such as osteoarthritis which presents a huge market opportunity for Paradigm's PPS treatment.

Subject to successful phase 2b results, the Company is aiming to achieve fast track designation and begin conducting a phase 3 trial in the US in 2019, completing in 2020. Successful phase 2b trials and fast track designation would be expected to generate significant big pharma interest.

In July 2017 the Company commenced a phase 2a clinical trial to treat people recently infected with the Ross River virus. The results of this trial are also expected to be released in mid-January 2019.

The Company continues to execute on its drug repurposing strategy. The key benefits of this strategy are lower costs, accelerated development timelines and higher success rates than the standard clinical development timeline.

To learn more please visit: www.paradigmbiopharma.com

For more information, please contact

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