

**Positive interim data from phase 2 rare disease trial presented at international medical congress.**

**KEY HIGHLIGHTS**

---

- Preliminary MPS-I phase 2 data presented at the 14th International Congress of Inborn Errors of Metabolism in Sydney.
  - PPS was well tolerated with no serious adverse events reported over a 24-week period.
  - Meaningful improvements in pain, function, and activities of daily living and an overall improvement in quality of life was observed in all patients.
  - Administration of PPS resulted in improvements in walk tests, range of motion and other standard tests of activities important to daily function of the patients.
  - Changes in the profile of biomarkers suggest PPS has the potential to modulate the inflammatory and joint degenerating biomarkers associated with arthralgia in MPS I patients.
- 

**Paradigm Biopharmaceuticals Ltd (ASX: PAR) (“Paradigm” or “the Company”)**, a clinical stage biopharmaceutical company focused on repurposing existing molecules for new indications with unmet clinical needs, is pleased to report preliminary data on the phase 2 pilot study of pentosan polysulfate sodium (PPS) for the treatment of mucopolysaccharidosis type I (MPS-I) that will be presented at the 14th International Congress of Inborn Errors of Metabolism in Sydney (ICIEM 2021). During the conference a poster detailing the promising data will be presented along with a discussion with the Head of the Metabolic Unit at the Adelaide Women and Children’s Hospital, Dr Drago Bratkovic, and Paradigm Global Head of Safety and MPS, Dr Michael Imperiale.

MPS-I is a rare disease caused by reduced levels, or the complete lack of, an enzyme responsible for the catabolism (break down) of glycosaminoglycans (GAG) resulting in the progressive accumulation of GAG in the tissues. The disorder causes problems with neurological, skeletal and cardiovascular development. There is no cure and children born with the most severe form of MPS-I do not typically survive beyond 10 years of age, without treatment. Current standard treatments include bone marrow transplant and enzyme replacement therapy to address the underlying cause of the disease.

The study at the Adelaide Women’s and Children’s Hospital, South Australia has enrolled three patients who are over halfway through the 48-week treatment regime. The encouraging data from these patients was presented at ICIEM 2021 by Dr Drago Bratkovic, Head of the Metabolic Clinic at the hospital.

The poster provides evidence PPS could help address the unmet medical needs of MPS-I patients and supported further studies.

### Summary highlights of the poster presentation included:

- PPS may address the unmet medical needs for patients who continue to experience pain and symptoms that affect function following treatment with the best current standard of care.
- PPS was well tolerated with no serious adverse events reported over a 24-week period.
- Meaningful improvements in pain, function, and activities of daily living and an overall improvement in quality of life was observed in all patients.
- Administration of PPS resulted in improvements in 2 and 6-minute walk tests, range of motion and other standard tests of activities important to daily function of the patients.
- Pharmacokinetic results demonstrated consistency in serum concentrations that were dose dependent.
- Changes in the profile of biomarkers suggest PPS has the potential to modulate the inflammatory and joint degenerating biomarkers associated with arthralgia in MPS-I patients.

**Paradigm interim Chief Executive Officer, Dr Donna Skerrett said,** *"We are very encouraged by the overall preliminary study data which supports our clinical development strategy for PPS as a viable treatment for children with residual musculoskeletal symptoms despite standard of care therapy in this difficult to treat disease."*

A phase 2 open-label study in up to 10 patients with MPS-I was initiated in September 2020. The primary aim of the study is to evaluate safety and tolerability of PPS over an initial 48-week period, with a 6-month treatment extension available, in patients treated with the current standard of care. Secondary and exploratory objectives include examining the effects of PPS on pain, function, and quality of life, pharmacokinetics, biomarkers, and inflammatory processes.

The open-label study remains ongoing with additional patient recruitment expected in CY22. Paradigm is currently exploring strategic partnerships to progress current and future clinical studies to further evaluate PPS as a treatment to address the critical unmet need of ongoing musculoskeletal symptoms in this very rare patient population.

Registered attendees can view the poster presentation via the ICIEM (<https://www.iciem2021.com.au/>), and after conclusion of the conference (23<sup>rd</sup> November) it will be available on the Paradigm website.

### About injectable PPS

Pentosan polysulfate sodium (PPS) is a medication that has been used in humans for over 60 years. Injectable PPS has previously been approved in European markets, where it is registered as an antithrombotic agent. In Australia, injectable PPS for human use is not currently available for sale. Injectable PPS is available via a Paradigm sponsored clinical trial or under the TGA Special Access Scheme to physicians for individual patients who satisfy strict criteria and is subject to approval from the TGA. Elmiron (the oral formulation utilised for interstitial cystitis) is the only PPS product approved in the US. A subcutaneous injectable formulation of PPS is currently being evaluated by Paradigm for the treatment of osteoarthritis and other inflammatory diseases in the US and other major global markets.

## About Paradigm Biopharmaceuticals

Paradigm Biopharmaceuticals LTD (ASX: PAR) is a late-stage drug development company with the mission to develop and commercialise pentosan polysulfate sodium for the treatment of pain associated with musculoskeletal disorders driven by injury, inflammation, ageing, degenerative disease, infection or genetic predisposition. Paradigm is also investigating proof-of-concept for the use of PPS in respiratory and heart failure indications.

## Forward Looking Statements

This Company announcement contains forward-looking statements, including statements regarding anticipated commencement dates or completions dates of preclinical or clinical trials, regulatory developments and regulatory approval. These forward-looking statements are not guarantees or predictions of future performance, and involve known and unknown risks, uncertainties and other factors, many of which are beyond our control, and which may cause actual results to differ materially from those expressed in the statements contained in this presentation. Readers are cautioned not to put undue reliance on forward-looking statements.

---

Authorised for release by the Paradigm Board of Directors.

Zilosul® is a registered Trademark of Paradigm Biopharmaceuticals Ltd (ASX: PAR).

To learn more please visit: [www.paradigmbiopharma.com](http://www.paradigmbiopharma.com)

### FOR FURTHER INFORMATION PLEASE CONTACT:

Simon White

Director of Investor Relations

Tel: +61 404 216 467

Paradigm Biopharmaceuticals Ltd

ABN: 94 169 346 963

Level 15, 500 Collins St, Melbourne, VIC, 3000, AUSTRALIA

Email: [investorrelations@paradigmbiopharma.com](mailto:investorrelations@paradigmbiopharma.com)