FOR PERSONAL USE ONLY

PARADIGM BIOPHARMACEUTICALS LIMITED

ASX RELEASE

13th February 2020

Paradigm presents MPS VI poster at the World MPS Symposium in Orlando, Florida

KEY HIGHLIGHTS

- **Paradigm has presented its MPS VI poster at the MPS World Symposium in Orlando, Florida.**
- **Poster presented outcomes of patient focus group which aimed to identify and validate clinical endpoints which produce the most clinically relevant endpoints for MPS subjects.**
- **Paradigm has focused on developing a Patient Centric Program for the forthcoming Phase 2/3 Pivotal trial into addressing the ongoing unmet need of residual musculoskeletal symptoms in patients who have received primary treatment for MPS.**
- **A Patient Centric approach is specifically tailored to the unmet clinical needs of MPS patients and will positively assist the Paradigm in Recruitment and Retention of MPS patients in the Phase 2/3 Clinical Trial.**
- **The FDA and EMA have agreed to a joint Parallel Scientific Advice Submission, the procedure commences in March.**

Paradigm Biopharmaceuticals Ltd (ASX: PAR) Paradigm is pleased to announce it has presented its MPS VI poster at the World MPS Symposium in Orlando, Florida. The research conducted by the Paradigm team has been used to identify clinically relevant endpoints to be discussed as part of the joint Scientific Advice Submission to the FDA (US) and the EMA (Europe) regulatory bodies. The procedure for the joint submission of the scientific advisory briefing documents commences in March 2020. Further information on the World MPS Symposium can be found at https://worldsymposia.org/.

Patient Centric Development

The objective of this research was to engage patients with MPS VI, and their caregivers in the drug development process to better understand the range of symptoms and the impact on function and activities of daily living (ADL). The methodology involved conducting a focus group with nine patients age 4-18 and their caregivers. An interactive forum with a series of open ended and polling questions were used to gain comprehensive understanding into the specific needs of patients suffering from the orphan disease.

The National MPS Society assisted Paradigm with recruitment of MPS VI patients and their caregivers to participate in the focus group.

FOR PERSONAL USE ONLY

The US FDA has published a Guidance for Industry entitled "Patient-Focused Drug Development: Collecting Comprehensive and Representative Input Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders". The aim of this patient-focused drug development is to "Enhance the Incorporation of the Patient's Voice into Drug Development" https://www.fda.gov/media/113653/download

MPS Participants were asked to rank 1st-4th most challenging; the first 2 most challenging are presented:

- 33.3% of patients/caregivers cited mobility and independence (each) as their most challenging, with over half citing mobility as most or 2nd most challenging;
- Fine motor tasks were reported by 78% as the most or 2nd most challenging;
- 33.3% of patients/caregivers cited sleep as most or 2nd most challenging.

The comprehensive understanding into the specific needs of MPS patients has enabled Paradigm to tailor its development program to the clinical unmet needs of MPS patients, providing strong clinical endpoints for the proposed Phase 2/3 trial. Paradigm believes this approach will also greatly assist in the recruitment and retention of patients in the proposed clinical trial and will likely improve market penetration of iPPS for treatment of MPS patients in conjunction with ERT as the company moves toward commercialisation.

Dr. Donna Skerrett, Paradigm's Chief Medical Officer, said:

"Improving the lives of MPS patients requires a deep understanding of their medical condition, experiences, needs and priorities of both patient and caregiver. The patient centric research conducted by the Paradigm team provides an opportunity to adopt and use these as a reference point for consistent patient engagement and to develop clinically meaningful endpoints which is especially important in orphan indications".

MPS VI

MPS VI is recognized as an orphan designation, and classified as a rare autosomal recessive, inherited lysosomal storage disorder caused by a deficiency of N- acetylgalactosamine 4–sulfatase, leading to accumulation of glycosaminoglycans (GAGs) in the lysosomes and physical manifestations. Current treatment for MPS patients includes Enzyme Replacement Therapy (ERT) which acts to reduce non-neurological symptoms and pain. MPS patients undergoing approved ERT however, continue to report ongoing stiffness, pain, inflammation, and heart and airway soft tissue manifestations.

Mr. Paul Rennie, Paradigm's Chief Executive Officer said:

"The research into the clinical unmet needs of MPS patients conducted by the Paradigm team and the presentation of the poster at the World MPS Symposium provides the company an important understanding of clinically meaningful end-points that will be incorporated into our clinical trial development program for iPPS in this orphan indication. This is an important step for Paradigm as we continue to work with key opinion leaders and MPS experts about how to achieve the best outcomes for patients. By having a trial design that patients see benefit from allows for better recruitment, retention and higher reimbursement potential should the trial be successful".

The presented poster can be viewed here

FOR PERSONAL USE ONLY

About injectable PPS (iPPS)

Injectable PPS (iPPS) is not currently registered in Australia, but it is was previously registered in four of the seven major global pharmaceutical markets. In those European markets, iPPS is registered as an antithrombotic agent. In Australia, iPPS for human use is not currently available for sale.

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

To learn more please visit: 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