Paradigm Biopharmaceuticals Ltd

11:54 13 Feb 2020

Paradigm Biopharmaceuticals presents patient-centric research at World MPS Symposium

Paradigm Biopharmaceuticals Ltd (ASX:PAR) has presented its poster presentation for MPS VI, a rare autosomal lysosomal storage disorder, at the World MPS Symposium in Orlando, Florida.

The company presented outcomes from a patient focus group which aimed to identify and validate clinical endpoints thus producing the most clinically relevant endpoints for MPS subjects.

Paradigm chief medical officer Dr Donna Skerrett 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".

Current treatment includes enzyme replacement therapy (ERT) which acts to reduce non-neurological symptoms and pain, however, many patients continue to report ongoing issues.

Paradigm is focused on repurposing the injectable drug pentosan polysulphate sodium (iPPS) to treat inflammation.

Clarifying patient needs
The objective of the company's 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.

A focus group was established with nine patients age 4-18 and their caregivers with a series of open-ended and polling questions to gain comprehensive understanding into the specific needs of patients suffering from the orphan disease.

MPS patients found the following activities most challenging:

- 33.3% of patients/caregivers cited mobility and independence as their most challenging;
- Fine motor tasks were reported by 78% as the most or second most challenging; and
- 33.3% of patients/caregivers cited sleep as most or second most challenging.

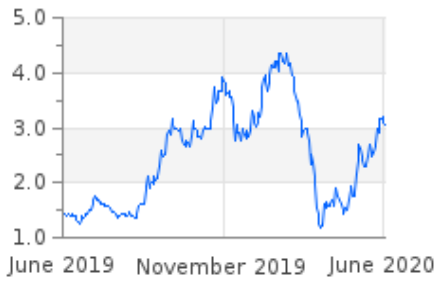
Patient-centric development
Paradigm chief executive officer Paul Rennie said: "The research into the clinical unmet needs of MPS patients conducted by the Paradigm team and the presentation of the poster at the World MPS Symposium provides the company an important understanding of clinically meaningful 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

Price: 3.07

Market Cap: $689.94 m

1 Year Share Price Graph



Share Information

Code:		PAR
Listing:		ASX
52 week	**High**	**Low**
	4.5	**1.08**

Sector: **Pharma & Biotech**

Website: **www.paradigmbiopharma.com**

Company Synopsis:
Paradigm Biopharmaceuticals Ltd (ASX:PAR) is listed on the Australian Securities Exchange.

action@proactiveinvestors.com.au

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 company believes this approach will not only assist in the recruitment and retention of patients for the trial but will likely improve market penetration of iPPS for treatment of MPS patients in conjunction with ERT - as the company moves toward commercialisation.

Scientific advice submission
The endpoints identified in the research will be discussed as part of the joint Scientific Advice Submission to the food and drug administration (FDA) USA and the European medicines agency (EMA) regulatory bodies.

The FDA and EMA have agreed to a joint parallel scientific advice submission, with the procedure to commence in March.