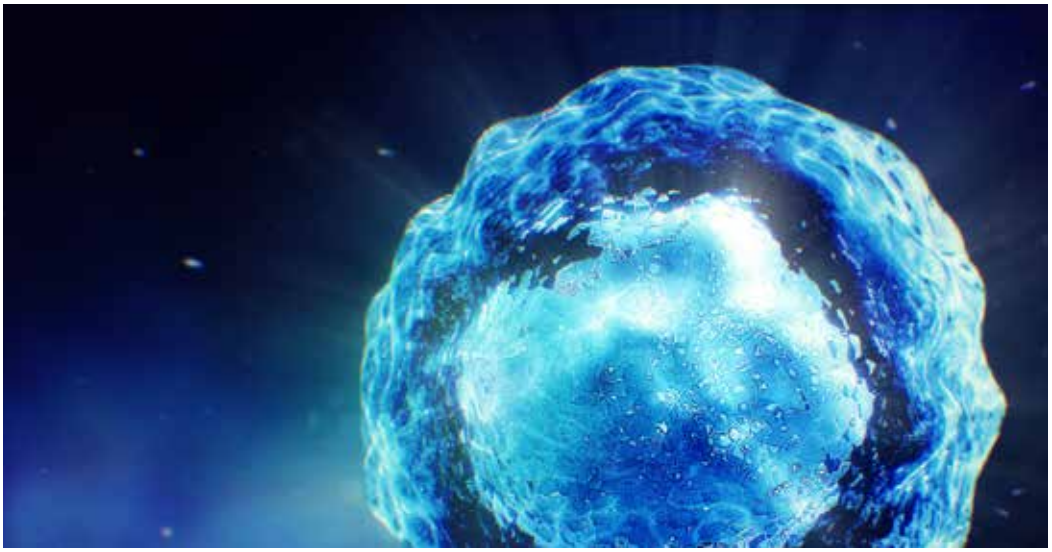
mesoblast



Mesoblast is a Global Leader in Innovative Cellular Medicines

Mesoblast (ASX:MSB; Nasdaq:MESO) has entered 2019 with the most mature cell therapy product pipeline and technology platform in the regenerative medicine industry.

Two commercial products have already been approved and marketed by the Company's licensees in Japan and in Europe.

Mesoblast has one product candidate which has successfully completed Phase 3 and has near-term commercial potential in the United States (U.S.), another product candidate having achieved clinical outcomes in line with the U.S. Food and Drug Administration (FDA) guidance for a registrable clinical indication for market authorization, and two additional Phase 3 assets with blockbuster potential.

Mesoblast's royalty income and milestone payments from licensees continues to grow. The Company has sufficient cash to achieve key commercial milestones, and access to additional non-dilutive sources of capital from strategic financial institutions whose extensive due diligence provides further third party validation of the strength of the product portfolio and patent estate.

Substantial Commercialization Opportunities

Lead product candidates under investigation are:

- Remestemcel-L for acute graft versus host disease (aGVHD)
- Revascor for advanced and end-stage heart failure
- MPC-06-ID for chronic low back pain due to degenerative disc disease

Additionally, Mesoblast has a promising emerging pipeline of products and next generation technologies.

Innovative technology

Mesoblast is developing immuno-selected, culture expanded cellular medicines based on mesenchymal precursor cells (MPCs) and their progeny, mesenchymal stem cells (MSCs). Rare mesenchymal lineage cells (approximately 1:100,000 of bone marrow cells) are found around blood vessels and are central to blood vessel maintenance, repair and regeneration. Preclinical studies have shown that these cells respond to damaged-tissue, secreting mediators that promote tissue repair and modulate immune responses. This mechanism of action enables the targeting of multiple disease pathways in a number of complex diseases where inflammation plays a central role and are resistant to conventional standard of care. A key feature of Mesoblast's patented mesenchymal lineage cells is that they are administered without the need for donor–recipient matching or recipient immune suppression, and therefore are often referred to as 'off-the-shelf' cellular medicines.

Diverse Portfolio of Advanced Cellular Medicines

Each of Mesoblast's product candidates has its own distinct technical characteristics, target indications, individual reimbursement strategy, separate commercialization potential and partnering opportunities.

Commercial and Late-Stage Product Pipeline

MARKETED

PLATFORM	PRODUCT	THERAPEUTIC AREA	APPROVAL	COMMERCIAL RIGHTS
MSC (Bone Marrow)	TEMCELL® HS Inj[1]	Acute Graft Versus Host Disease — 1st allogeneic regen med approved in Japan	✓	JCR Japan
MSC (Adipose)	Alofisel®[2]	Perianal Fistula — 1st allogeneic regen med approved in Europe	✓	Takeda Global

IN DEVELOPMENT

TIER 1

PLATFORM	PRODUCT CANDIDATE	THERAPEUTIC AREA	PRE-CLINICAL / PHASE 2 / PHASE 3	COMMERCIAL RIGHTS
MSC	Remestemcel-L	Acute Graft Versus Host Disease	Phase 3	mesoblast
MPC	Revascor	Advanced HF (Class II & III)	Phase 3	mesoblast
		End-Stage HF (Class III &IV)[3]	Phase 3	TASLY China[4]
MPC	MPC-06-ID	Chronic Low Back Pain	Phase 3	mesoblast
MPC	MPC-300-IV	Rheumatoid Arthritis	Phase 2	mesoblast
		Diabetic Nephropathy	Phase 2	

TIER 2

Includes ***Remestemcel-L*** (Crohn's disease – biologic refractory), ***MPC-25-IC*** (Acute Cardiac Ischemia), ***MPC-25-Osteo*** (Spinal Fusion) and ***MPC-75-IA*** (Knee Osteoarthritis)

1 Mesoblast receives royalty income from its licensee JCR Pharmaceuticals Co Ltd on sales of JCR's TEMCELL® Hs. Inj. product in Japan.
2 Mesoblast will receive royalty income from its licensee Takeda Pharmaceuticals on Takeda's worldwide sales of its product Alofisel® in the local treatment of perianal fistulae.
3 Study funded by the United States National Institutes of Health (NIH) and the Canadian Health Research Institute; conducted by the NIH-funded Cardiothoracic Surgical Trials Network.
4 Tasly's rights are limited to China; Tasly also has rights to develop MPC-25-IC for Acute Cardiac Ischemia.

This chart is figurative and does not purport to show individual trial progress within a clinical program.

Acute Graft Versus Host Disease, a Life-threatening Inflammatory Condition

Mesoblast plans to initiate the FDA process of filing a Biologics License Application (BLA) for market authorization of remestemcel-L in the U.S. where there are no approved therapies for steroid-refractory aGVHD. Underpinning Mesoblast's confidence in the U.S. market access plan is the Japan experience, where Mesoblast's licensee, JCR Pharmaceuticals, markets TEMCELL®[1] HS Inj. for children and adults with aGVHD. Importantly, TEMCELL has achieved substantial adoption rates in less than three years, helping inform the view of the product value proposition potential in the U.S. market. To ensure a successful product launch, Mesoblast is establishing a focused sales team that will target the principal U.S. transplant centers, and will be in place on FDA approval.

Revascor for Advanced and End-stage Heart Failure

Mesoblast is developing Revascor, which comprises 150 million MPCs, to fill the treatment gap for both advanced and end-stage chronic heart failure. The objective is to use Revascor to prevent or delay further progression of heart failure or cardiac death in patients who are no longer responsive to maximal standard of care heart failure drugs.

Advanced Heart Failure

Mesoblast has completed enrollment of 566 patients in the Phase 3 trial evaluating Revascor in patients with moderate-to-severe advanced chronic heart failure, a progressive disease of cardiac inflammation. The placebo-controlled trial will complete when sufficient primary endpoint events have accrued. In April 2017, the Phase 3 trial was successful in a pre-specified futility analysis of the primary efficacy endpoint in the first 270 patients.

In the U.S. alone, there are more than 1.3 million patients with New York Heart Association (NYHA) class III chronic heart failure who have high rates of morbidity and mortality despite existing therapies. The major unmet medical need in these patients represents a potential multi-billion dollar market opportunity for Mesoblast.

End-Stage Heart Failure

In the first half of 2019, Mesoblast plans to meet with the FDA to discuss a potential approval pathway following the clinically meaningful outcomes of reduction in major gastrointestinal (GI) bleeding and related hospitalizations seen in a Phase 2 trial of Revascor in patients with end-stage heart failure and a left ventricular assist device (LVAD). This potentially life-threatening complication is the most common non-surgical complication in LVAD recipients and occurs in up to 40% of patients. The trial was sponsored by the U.S. National Institutes of Health (NIH).

In this Phase 2 trial of Revascor, a single injection of Revascor administered directly into the heart resulted in a 76 percent reduction in major gastrointestinal (GI) bleeding events and in a 65 percent reduction in associated hospitalizations. This suggests that Revascor reversed endothelial dysfunction which is responsible for the abnormal vasculature in the GI tract and severe bleeding in LVAD patients.

Reduction in GI bleeding and associated hospitalizations in a previous 30-patient pilot trial of Mesoblast's MPCs were the basis of the Regenerative Medicine Advanced Therapy (RMAT) designation granted in December 2017 by the FDA for use of Revascor in LVAD patients. In a subsequent meeting in 2018, the FDA advised Mesoblast that the defined endpoint of reduction in major GI bleeding and rehospitalization is an appropriate clinically meaningful endpoint and could be the basis of an approved indication for use of Revascor given the life-threatening nature of the condition, and the RMAT designation under which Revascor is being regulated.

Mesoblast has entered into a strategic cardiovascular partnership with Tasly Pharmaceutical Group for China. Tasly is China's leading cardiovascular company. Tasly plans to meet with the National Medical Products Administration of China in the first half of 2019 to discuss the regulatory approval pathway for Revascor in China. Tasly and Mesoblast will leverage each other's clinical trial results in China, the U.S. and other territories to support their respective regulatory submissions.

Chronic Low Back Pain Due to Inflammatory Degenerative Disc Disease

In the U.S., the declared opioid public health emergency and significant associated mortality has brought additional attention to Mesoblast's product candidate, MPC-06-ID, with the Phase 3 trial completing enrollment of 404 patients in March 2018. More than half of the prescriptions for opioids are for people seeking relief from chronic low back pain. There is a desperate need for a therapy that can offer both a durable reduction in pain and improvement in function without the risk of opioid addiction.

Underpinning Mesoblast's confidence that MPC-06-ID may meet this medical need are the Phase 2 data outcomes in 100 patients that supported the ongoing Phase 3 trial which showed that a single intra-discal injection of MPC-06-ID alleviated pain and improved function for up to three years in patients whose symptoms were not adequately treated with current standard of care therapies. The population suffering from chronic low back pain due to intervertebral disease is estimated at more than 3.2 million patients in the U.S. alone.

Scalable Manufacturing

The inherent technical properties of Mesoblast's mesenchymal lineage cells allow for scalable culture expansion to produce anticipated commercial quantities with batch to batch consistency and reproducibility. Proprietary media formulations, advances in development of 3D bioreactor technology and automation are intended to deliver step-changes improvement in product yield. Clinical supplies and anticipated commercial manufacturing requirements will be produced by a specialized, contract manufacturing organisation under applicable Good Manufacturing Practices (GMP).

Robust Intellectual Property Estate

Mesoblast's intellectual property portfolio encompasses approximately 800 patents or patent applications across 69 patent families, which the Company believes will provide substantial competitive advantages for the commercial development of its cell-based therapies in major markets including the U.S., Europe, Japan and China.

Lonza's cell manufacturing facility in Singapore.

Evidence-based Science and Translational Medicine

Mesoblast's approach to product development is to ensure rigorous scientific investigations are performed with well-characterized cell populations in order to understand mechanisms of action for each potential indication. Extensive preclinical translational studies guide clinical trials that are structured to meet stringent safety and efficacy criteria set by international regulatory agencies. All trials are conducted under the continuing review of independent Data Safety Monitoring Boards comprised of independent medical experts and statisticians. These safeguards are intended to ensure the integrity and reproducibility of results, and to ensure that outcomes observed are scientifically reliable.

Markets Opportunities for Lead Products

Acute Graft Versus Host Disease

- >30,000 allogeneic bone marrow transplants performed globally (>20K US/EU) annually, ~20% pediatric
- Steroid-refractory aGVHD represents >USD $700m U.S./EU market opportunity[7]

Advanced Heart Failure

- U.S. healthcare costs for NYHA class II-IV patients $115bn/year
- Hospitalizations account for ~69% of expenditure
- Multi-billion dollar annual market opportunity in in the U.S.

End-stage Heart Failure with a Left Ventricular Assist Device

- 4,500 – 5,500 assist devices are implanted annually in the U.S.
- U.S. LVAD market is growing double-digit CAGR and represents significant market growth opportunity

Chronic Low Back Pain Due to Disc Degeneration

- MPC-06-ID development focused on over ~3.2m patients in U.S. alone
- U.S. market opportunity >USD $1 billion

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