

# Financial Tear Sheet

## Download Mesoblast Corporate Backgrounder

[Mesoblast Corporate Backgrounder \(350 KB\)](#)

Mesoblast is a world leader in developing innovative cellular medicines.

The Company has established what it believes is the industry's most clinically advanced and diverse portfolio of cell-based product candidates with three programs in Phase 3 clinical studies. Consistent and durable efficacy data have been demonstrated across multiple difficult-to-treat diseases.

Lead product candidates under investigation are:

- MSC-100-IV for acute graft versus host disease
- MPC-150-IM for advanced heart failure
- MPC-06-ID for chronic low back pain due to disc degeneration
- MPC-300-IV for biologic refractory rheumatoid arthritis and diabetic nephropathy

Mesoblast also has a strong emerging pipeline of products for follow-on indications.

The Company has leveraged its proprietary technology platform, which is based on specialized cells known as mesenchymal lineage adult stem cells, to establish a broad portfolio of late-stage product candidates.

Its allogeneic, 'off-the-shelf' cell product candidates are being evaluated in a range of advanced-stage diseases with high, unmet medical needs, including cardiovascular diseases, musculoskeletal disorders, immunologic conditions and oncologic and hematologic diseases.

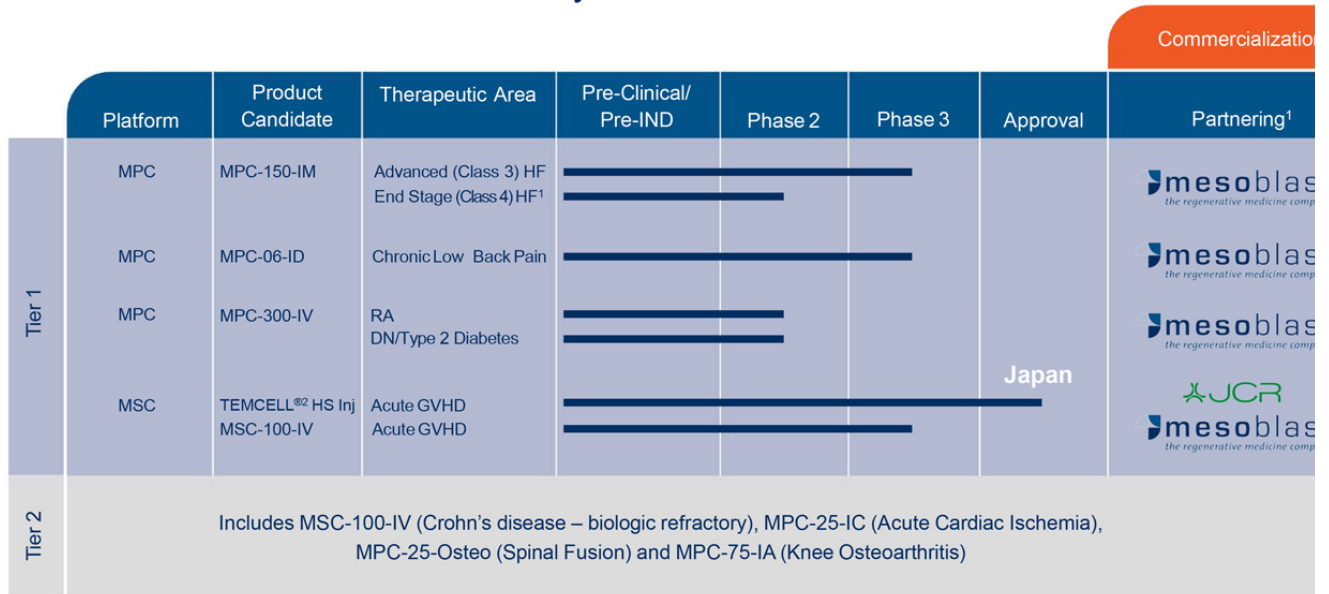
It is Mesoblast's goal to have the first industrially manufactured allogeneic stem cell product approved in the United States with its product candidate for the treatment in children of acute graft versus host disease complication of a bone marrow transplant. Mesoblast's licensee in Japan, JCR Pharmaceuticals Co. Ltd., is marketing its mesenchymal stem cell-based product for the treatment of this disease in children and HS Inj. was the first allogeneic cellular medicine to receive full approval in Japan.

## Prioritized Portfolio of Clinically Distinct and Advanced Product Candidates

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

The majority of time and resources are focused on developing Tier 1 product candidates. Tier 2 programs are also in development and may advance to Tier 1 depending on the merit of newly generated data, and other partnering options.

## Portfolio of Advanced Product Candidates: Well Positioned for the 21<sup>st</sup> Century Cures Act Environment



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

1. Clinical trial is fully funded by the U.S. National Institutes of Health and the Canadian Institute of Health Research
2. TEMCELL<sup>®</sup> HS. Inj. is a registered trademark of JCR Pharmaceuticals Co., Ltd.

## Lead Product Candidates under Clinical Investigation

**MSC-100-IV** is a Phase 3 intravenously-delivered product candidate that is being developed for the treatment of acute graft versus host disease following allogeneic bone marrow transplantation. An open label MSC-100-IV in children is being conducted to support filing of a biologic license application to the United States Food and Drug Administration (FDA) for regulatory approval. This Phase 3 trial has completed and is aimed at building on results from an Expanded Access Program where 241 children were treated with MSC-100-IV for severe, multi-line refractory acute graft versus host disease. Clinically meaningful responses and increased survival were demonstrated.

In late 2016, the Phase 3 trial was successful in a pre-specified interim futility analysis. The FDA has granted a Fast Track designation for the use of MSC-100-IV to improve overall response rate in children acute graft versus host disease. Fast Track designation has the potential to shorten the time to FDA approval through priority review and a streamlined rolling review process

Mesoblast plans to evaluate the use of MSC-100-IV in adult patients with high-risk steroid-refractory acute graft versus host disease.

**MPC-150-IM** is a late-stage cardiovascular product candidate for patients with advanced and end-stage heart failure. The mechanism of action by which MPC-150-IM is thought to exert its effects in these patients through secretion of potent biomolecules which reduce damaging inflammation and strengthen the native heart by induction of a mature blood vessel network.

Approximately half of people who develop heart failure die within five years of diagnosis. Patients with advanced chronic heart failure (defined as New York Heart Association Class III or Class IV) have the disease, recurrent hospitalizations and mortality. A Phase 3 trial is enrolling patients with chronic heart failure across North America. The trial's primary efficacy endpoint is a comparison of recurrent non-fat major adverse cardiac events between either patients treated with MPC-150-IM or sham-treated controls. In April 2017, this trial achieved a successful pre-specified interim futility analysis of the efficacy of patients of the 600-patient trial. Based on this outcome, an independent data monitoring committee formally recommended the continuation of the trial.

Enrollment has been completed in a Phase 2b trial sponsored by the United States National Institutes of Health and the Canadian Institute of Health Research evaluating MPC-150-IM in 159 patients with or without requiring a left ventricular assist device (LVAD). In this patient population, despite optimal medical therapy, one-year mortality is around 30%.

The FDA has granted Regenerative Medicine Advanced Therapy (RMAT) designation for Mesoblast's mesenchymal precursor cell therapy in the treatment of heart failure patients with left ventricular assist devices (LVADs). The RMAT designation under the United States 21st Century Cures Act aims to expedite the development of regenerative medicine therapies intended for the treatment of serious diseases and life-threatening conditions.

The basis of this RMAT designation grant came from the completed study data set of a 30-patient randomized, blinded, placebo-controlled pilot trial of Mesoblast's MPCs at a dose of 25 million cells in heart failure patients with LVADs, and related analyses. These preliminary clinical data suggest that Mesoblast's MPC product improved native heart function, prolonged the time post LVAD implantation of a first hospitalization for gastrointestinal bleeding event, and improved early survival rates in these LVAD recipients.

**MPC-06-ID** is a non-opioid alternative for the treatment of chronic low back pain due to disc degeneration. When disc degeneration has progressed to a point that pain and loss of function can no longer be managed by medication and physiotherapy, major invasive surgery such as spinal fusion is the only remaining option. Existing therapies treat the symptoms of the disease, but are not disease-modifying or address the underlying disease cause. As a result, the Company believes the most significant unmet need is a therapy that can not only improve the patient's pain and function, but has the ability to reverse, halt or slow the progression of the disease.

Phase 2 results demonstrated that a single injection of MPC-06-ID resulted in meaningful improvements in both pain and function that were durable for at least 36 months. By treating the cause of chronic low back pain, MPC-06-ID could fill an unmet treatment gap for a large population of patients. A Phase 3 trial of 360 patients is actively enrolling patients in the United States and Australia.

**MPC-300-IV** is an intravenously-delivered immunomodulatory product candidate for the treatment of chronic inflammatory conditions, including biologic-refractory rheumatoid arthritis, a chronic autoimmune disease with an etiology that affects approximately one percent of the population. The disease is attributed to chronic inflammation affecting the synovial membrane of multiple joints, which eventually leads to cartilage and bone damage and health-related quality of life in patients with rheumatoid arthritis is significantly impaired by pain, fatigue, and decline in musculoskeletal function. It is also associated with an increased risk of cardiovascular disease.

The rheumatoid arthritis population resistant to anti-tumor necrosis factor agents constitutes about one-third of patients treated with these agents. The goal of therapy in these patients is to achieve early and sustained remission and activity which correlates with prevention of structural joint damage in rheumatoid arthritis.

In a 48-patient randomized, placebo-controlled Phase 2 trial in biologic refractory rheumatoid arthritis a single intravenous infusion of MPC-300-IV was well tolerated, without serious adverse events, and demonstrated dose-related improvement in clinical symptoms, physical function, and disease activity relative to placebo through the 12-week primary endpoint and durability through the 39-week follow-up period of this study.

#### **MLC Technology Platform**

Mesoblast's proprietary mesenchymal lineage cells are central to blood vessel maintenance, repair and regeneration, largely via the secretion of growth factors that act on neighbouring endothelial cells to promote regeneration and function. These rare cells (approximately 1:100,000) are found around blood vessels and respond to signals associated with tissue damage, secreting mediators that promote tissue repair and immune responses.

The Company's 'off-the-shelf' product candidates are allogeneic and immune-privileged, meaning cells from one donor may be used in many different recipients without the need for tissue matching.

#### **Mesoblast's proprietary cells have two distinct technical properties:**

**Expansion** - proprietary methods have been developed that enable the large-scale expansion of mesenchymal lineage cells while maintaining their ability to produce key biomolecules associated with tissue healing. This allows production of a cellular product with consistent, well-defined therapeutic properties, batch release criteria and established potency assays, with accompanying manufacturing economies of scale.

**Immune Privilege** – mesenchymal lineage cells are immune privileged, meaning they do not initiate and facilitate an immune response when administered to unrelated patients. Based on extensive preclinical studies, the immune response that contribute to the lack of immune response appear to be multi-factorial and involve lack of immune stimulatory molecule expression, as well as release of various biomolecules involved in the inhibition of immune response.

Mesenchymal lineage cells can be taken from healthy young adult donors from various tissue sources, including bone marrow, fat tissue and dental pulp. Mesoblast isolates these cells from bone marrow and dental pulp master cell banks, which are then further amplified to generate thousands of therapeutic doses without the need for tissue matching. Each allogeneic product candidate is packaged into vials, frozen, and then transported to facilities for use in clinical trials worldwide.

#### **Robust Intellectual Property Position**

Mesoblast's intellectual property portfolio encompasses approximately 800 patents or patent applications across 69 patent families, which the Company believes will provide substantial competitive advantage and development of its cell-based therapies in major markets including the United States, Europe, Japan and China. These cover compositions of matter and uses for mesenchymal lineage-based technologies and regenerative medicine product candidates and technologies, as well as for elements of proprietary manufacturing processes.

#### **World Class Stem Cell 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 potent product. Preclinical translational studies guide clinical trials that are structured to meet stringent criteria set by international regulatory agencies.

#### **Strategic Alliances**

Mesoblast has established strategic relationships with several industry leaders to support clinical development, manufacturing and commercial capabilities.

JCR Pharmaceuticals Co. Ltd has been granted an exclusive right in Japan to Mesoblast's mesenchymal stem cell technology for use in conjunction with the treatment of hematologic malignancies using hematopoietic stem cell transplants derived from peripheral blood, cord blood or bone marrow.

An alliance with the Lonza Group was established to ensure long-term commercial manufacturing requirements of our products. The Company believes this alliance provides Mesoblast with significant commercial advantages including capacity to meet long-term global supply of its proprietary product candidates, dedicated access to Lonza's cell therapy facilities in Singapore, and the potential for a purpose-built manufacturing facility in Lonza to meet Mesoblast's long-term commercial objectives.

#### **Experienced Management Team**

Led by Chief Executive Dr Silviu Itescu, a pioneer of cellular medicines, Mesoblast employs approximately 80 employees in the United States, Australia and Singapore.

#### **Corporate**

Mesoblast was publically listed on the Australian Securities Exchange (ASX:MSB) in December 2004. The Company has a Level 3 American Depositary Receipt program facility and listed on the Nasdaq (NASDAQ) in the United States in November 2015.

Stock Performance

MSB.AX (Common Stock)	
Exchange	NASDAQ GS (US Dollar)
Price	<b>\$5.48</b>
Change (%)	0.00 (0.00%)
Volume	1
52 Week High	\$8.78
52 Week Low	\$4.74
Market Cap	\$2,625,685,304
Rolling EPS	-0.38
PE Ratio	0
Shares Outstanding	479,139,654

Data as of 12 November 2018 4:00 p.m. ET



Company Announcements

13 November 2018	<a href="#">LVAD Results Webcast Presentation</a>
12 November 2018	<a href="#">LVAD Trial Results Provide Potential Regulatory Pathway</a>
12 November 2018	<a href="#">Pause in Trading</a>

SEC Filings

Filing Date	Form
07 November 2018	<a href="#">6-K</a>
31 October 2018	<a href="#">6-K</a>
17 October 2018	<a href="#">6-K</a>
04 October 2018	<a href="#">CT ORDER</a>

Data provided by Nasdaq. Minimum 15 minutes delayed.

Upcoming Events

Date	Title
13 November 2018	<a href="#">Phase 2b Results in End-stage H</a>

Corporate Governance

<a href="#">Silviu Itescu, MBBS, FRACP</a>	C
<a href="#">Paul Hodgkinson, MA (Hons), FCA</a>	C
<a href="#">Roger D. Brown, BA</a>	S
<a href="#">Lee Golden, MD</a>	C
<a href="#">Peter Howard, BSc, LLB (Hons)</a>	C
<a href="#">Sue MacLeman, BPharm, MMktg, MLaw, FACPP, FAICD</a>	C
<a href="#">John McMannis, PhD</a>	M
<a href="#">Julie Meldrum, Inv Rel (AIRA)</a>	G
<a href="#">Michael Schuster, MS, MBA</a>	Ir
<a href="#">Karen Segal, PhD</a>	D
<a href="#">Paul Simmons, PhD</a>	R
<a href="#">Donna Skerrett, MD, MS</a>	C
<a href="#">Jonathan R. Symonds, CBE, BA, FCA</a>	C
<a href="#">Darin Weber, PhD</a>	R