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the regenerative medicine company

2014 Half-Year Financial Results & Corporate Strategy

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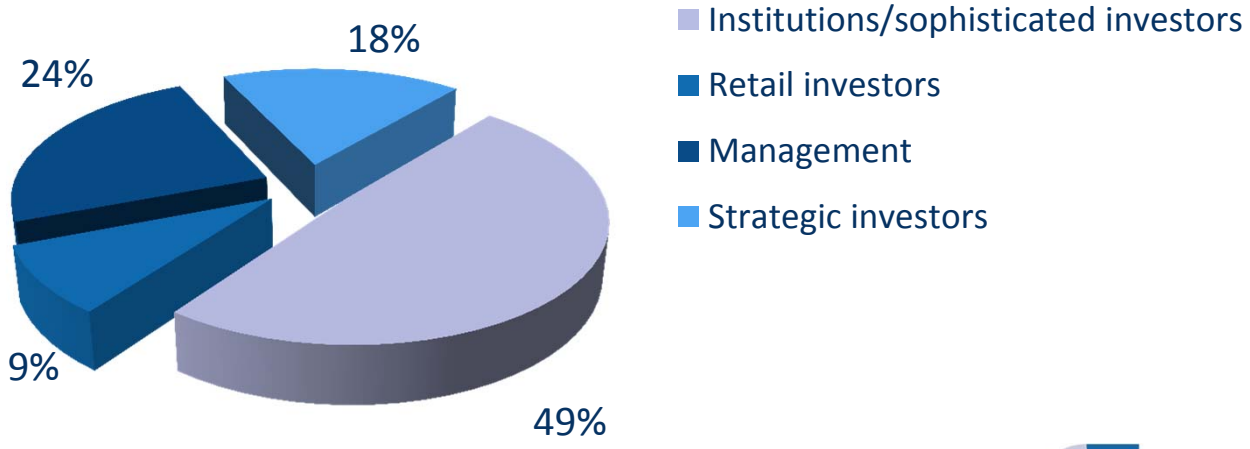
Shareholder value and ownership

Market valuation	
Issued shares	321 million
Current share price (market close, 25 February 2014)	$5.91
Market capitalization, circa	$1.89 billion
Cash reserves (at 31 December 2013)	$250 million

Shareholder ownership



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Cash

CASH RESERVES	31 December 2013 $m	30 June 2013 $m	Half-Year Movement $m
Cash reserves	**250.3**	**315.3**	**(65.0)**

CASH FLOWS	Dec 2013 $m	Dec 2012 $m	Movement $m
Net cash outflows	**67.6**	**26.8**	**40.8**
Extract of items included in Net cash outflows (above):			
Cash outflow from operations activities	***48.0***	***25.0***	***23.0***
Cash outflow from investing activities	***21.2***	***3.4***	***17.8***

- Mesoblast's strong cash balance allows us to bring multiple MPC products within our pipeline to market whilst our acquisition of late-stage MSC assets provides opportunity for earlier product launch and revenues.
- Movement in cash flow from operations for the Dec 2013 half-year increased by $23.0m on the comparative period predominately due to an increased level of operational activity in clinical trials, cell production and support.
- Movement in cash flow from investing activities for the Dec 2013 half-year increased by $17.8m on the comparative period mostly due to the one-off acquisition of the culture-expanded MSC therapeutic business.

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Results – P&L

Loss after tax	Dec 2013 $m	Dec 2012 $m	Movement $m
Revenue & other income	**20.8**	**14.7**	**6.1**
Expenses from operations	**51.6**	**42.5**	**9.1**
Made up of:			
Research & development	20.7	21.6	(0.9)
Manufacturing commercialization	13.2	9.1	4.1
Management & administration	17.7	11.8	5.9
Share-based payments expense	**5.3**	**6.6**	**(1.3)**
Net loss after tax	**30.9**	**27.8**	**3.1**

- The increase in revenue is principally due to research and development tax incentive revenue recorded in the current half-year of $5.8m.
- The increase in expenditures is a result of the following:
  - increased clinical trial activities in type 2 diabetes, end-stage kidney disease, and rheumatoid arthritis;
  - increased product manufacturing to support these programs as well as producing material needed to conduct the phase 3 congestive heart failure trial, inventory supply and bioreactor process development; and
  - acquisition of the culture-expanded MSC therapeutic business and related patent portfolio;

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2014 financial half-year highlights

- Strong cash reserves of $250.3 million at 31 December 2013.
- Positive results at 12 months in a 100 patient Phase 2 trial for lumbar intervertebral disc repair evaluating safety and efficacy of MPCs.
- United States Food and Drug Administration (FDA) clearance of Phase 3 trial of MPCs for chronic congestive heart failure within the minimum 30-day period. This 1,700 patient trial is being funded and sponsored by our clinical and commercial development partner, Teva Pharmaceutical Industries Ltd., with recruitment across multiple United States sites.
- Positive topline efficacy results from Phase 2 trial of MPCs in patients with type 2 diabetes with a mean disease duration of 10 years and inadequate glucose control by Metformin and other oral glucose-lowering agents.
- Commenced a Phase 2 trial in Australia to evaluate the effects over three months of a single infusion of one of two MPC doses in 30 patients with type 2 diabetes and end stage kidney disease.

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2014 financial half-year highlights (continued)

- Acquired culture-expanded MSC assets, resulting in broadening of market opportunities with additional Phase 3 programs, accelerated commercial product launch, and strengthening of leadership position in regenerative medicine.
- Mesoblast collaborator, JCR Pharmaceuticals, announced plans to submit for registration in Japan use of MSCs in the treatment of steroid-refractory graft versus host disease in children and adults after bone marrow transplant.
- Strengthened MPC intellectual property estate and acquired culture-expanded MSC intellectual property, complementing and extending Mesoblast's existing patent estate. Key patents for MPCs were also granted in Europe, Japan and China.
- Optimized manufacturing operations at Singapore and United States plants.
- Ongoing optimization of bioprocessing to support commercial product scale-up.

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Corporate strategy

- Our principal objective is to bring multiple cell-based products to market within a parallel timeframe

- To achieve our objectives we rely on maintaining our

  - technological leadership position,
  - financial strength,
  - robust manufacturing operations,
  - delivery of late-stage clinical programs, and
  - formation of strategic partnerships to enhance commercial success

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Mesoblast proprietary core technologies

- Culture-expanded Mesenchymal Stem Cells (MSCs) being developed to induce rapid clinical responses following frequent multi-dose administration systemically
- Highly purified, immunoselected Stro-1/Stro-3 positive Mesenchymal Precursor Cells (MPCs) being developed to generate more durable clinical outcomes following single-dose or intermittent administration locally or systemically
- Ongoing development of therapeutic applications of Dental Pulp Stem Cells (DPSCs) and Expanded Hematopoietic Stem Cells (HSCs)
- Product lifecycle extension developing next generation ‘modified’ stem cell therapies

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Key strategic alliances

- Mesoblast has established and intends to increase strategic alliances to facilitate its corporate strategy of bringing multiple products to market within a parallel timeframe

- Alliances to date include:

  – cardiovascular and neurological programs using MPCs in partnership with **Teva Pharmaceutical Industries Ltd**

  – use of MSCs in Graft Versus Host Disease programs in children and adults in Japan in partnership with **JCR Pharmaceuticals**

  – biologics manufacturer **Lonza** to ensure commercial scale-up and supply, product delineation, COGS reductions, and expansion of clinical manufacturing

Robust intellectual property estate

- Ownership of or exclusive rights to more than 60 patent families which provide major commercial advantages and long-term protection across mesenchymal cell lineage platforms, including immunoselected and purified MPCs and culture-expanded MSCs

- Additional patent families cover compositions-of-matter or uses of DPSCs, expanded HSCs and SDF-1 technologies

- In addition to compositions-of-matter, IP covers efficient processes for cell extraction, purification and manufacturing scale-up, and the broad range of applications for which our technologies are being developed in key markets including the United States, Europe, Japan and China

- Together, Mesoblast's intellectual property and state-of-the-art manufacturing processes facilitate the Company's commercial model to develop cost-effective biotherapeutics across multiple clinical indications

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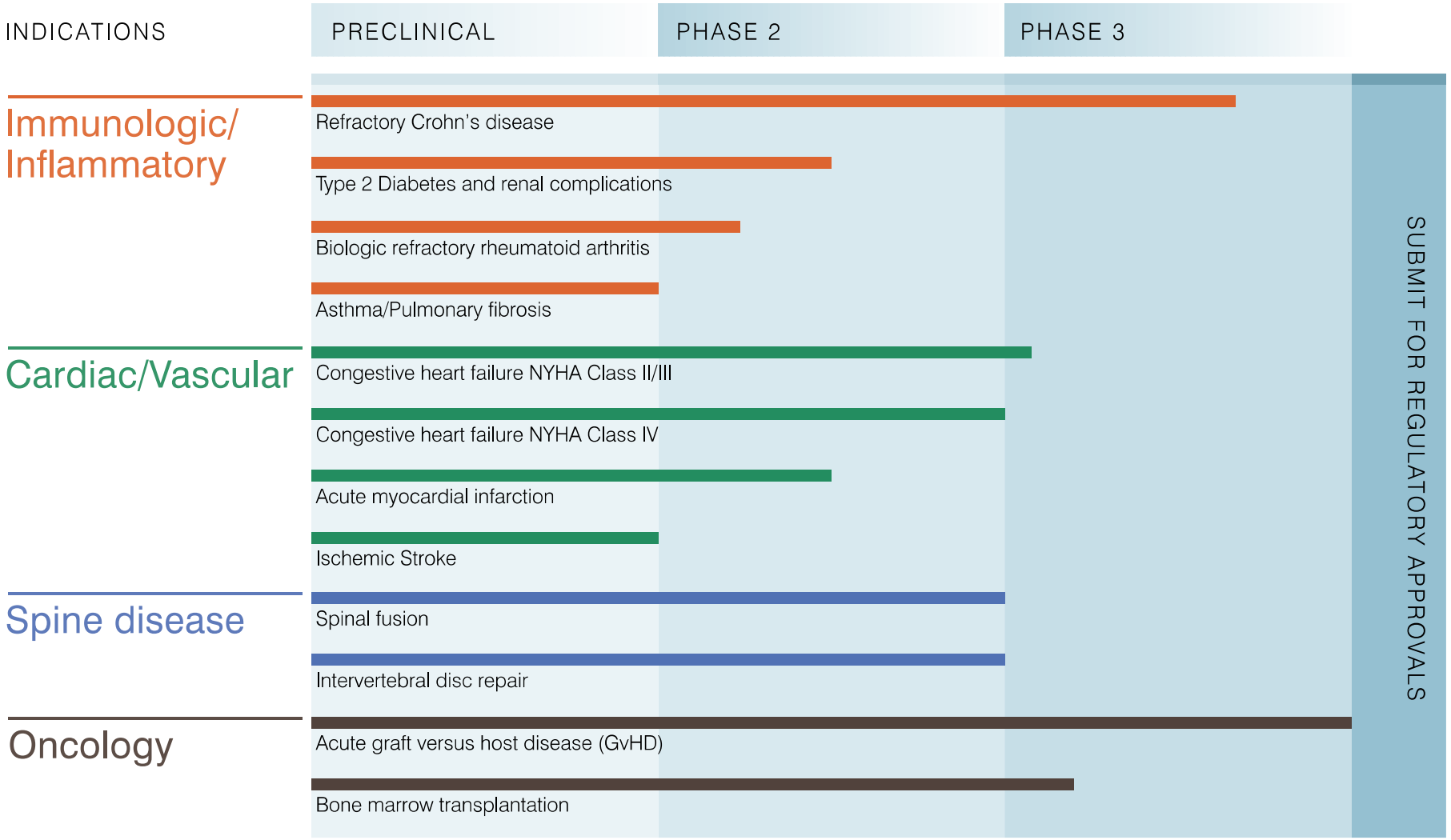
Mechanisms of Action (MOA) Drives Product Development

Mesoblast's strategic product focus is guided by specific MOAs of our cellular therapies.

The mesenchymal lineage stem cells respond to signals from inflammation and /or tissue damage by releasing a range of factors which:

- stimulate blood vessel growth and maturation, and reverse endothelial dysfunction
- increase survival and improve function of cardiac muscle cells, cells of central nervous system, bone-forming cells, and cartilage-producing cells
- tissue remodelling by reduction in scar formation and fibrosis
- induce polarization of pro-inflammatory monocytes to a non-inflammatory phenotype;
- inhibit activated T cells and induce regulatory T cells

Broad pipeline in key therapeutic areas



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Therapeutic areas of focus

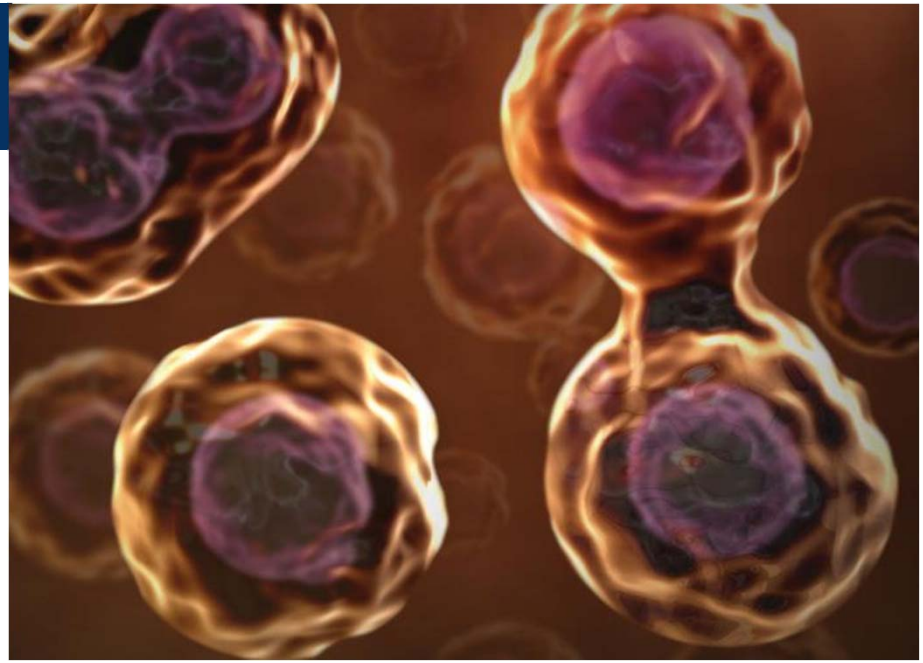
1. Programs in inflammatory and immune-mediated conditions

Phase 3

- Crohn's disease

Phase 2

- Type 2 diabetes inadequately controlled by Metformin and other oral glucose-lowering agents
- Chronic kidney disease with type 2 diabetes
- Rheumatoid arthritis



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Phase 3 program - Crohn's disease

- A 330-patient Phase 3 trial of Prochymal® (remestemcel-L, human mesenchymal stem cells for intravenous infusion) in patients with refractory moderate to severe Crohn's disease (CD) is underway
- The MOA is believed to be mediated through modulating the 'hyperactive state' of the immune system in particular down regulating pro-inflammatory actions of Th17 cells, IL-17 levels, and induction of Fox P3 regulatory T cells
- CD is a chronic inflammatory condition of the gastrointestinal tract that can cause pain, hospitalizations, bowel perforation, severe infection and death
- The Phase 3 trial is targeting CD patients who are unresponsive, refractory or intolerant to existing treatments including corticosteroids, immunosuppressants and biologics.
- This group represents a significant unmet medical need with over 700,000 people in the US have Crohn's disease, with 20,000 new cases diagnosed annually
  - Approx. 60,000 patients in the US alone are intolerant, unresponsive or refractory to existing biologics (e.g. TNF alpha inhibitors)
- The Phase 3 trial successfully met a pre-defined interim analysis for futility undertaken after 207 patients were enrolled

Type 2 diabetes

- Type 2 diabetes is a disease of excessive weight and dietary overload associated with a state of systemic inflammation
- pro-inflammatory cytokines such as IL-6 and TNF-alpha produced by inflammatory monocytes, called M1 type monocytes, and by fat cells or adipocytes, induce insulin resistance
- Diabetic kidney and liver diseases are associated with persistent monocyte inflammation and do not respond to glucose-lowering agents
- Mesenchymal lineage stem cells secrete factors that polarize pro-inflammatory M1 type monocytes to an anti-inflammatory M2 type
- MPC administration is being evaluated as an immunomodulatory treatment for the kidney

Type 2 diabetes (continued)

- Completed enrollment of 60-patient Phase 2 safety study evaluating single intravenous MPC injection in patients with type 2 diabetes inadequately controlled on Metformin with or without other oral glucose-reducing agents

- Top-line results showed:
  - No treatment-related adverse events
  - A dose dependent reduction in hemoglobin A1c (HbA1c) at 8-12 weeks after a single MPC infusion
  - Significant increase in the number of patients at target HbA1c <7% at week 12 following a single infusion of the highest MPC dose compared with placebo

- HbA1c is the accepted clinical standard and the primary endpoint in clinical trials for long-term (e.g. three months) glycemic control in subjects with type 2 diabetes

- *These results suggest that immunomodulatory effects of a single MPC injection may improve glucose control on top of Metformin*

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Therapeutic areas of focus

2. Programs in cardiac and vascular conditions

Phase 3

Congestive heart failure NYHA Class II/III – using MPCs

Phase 2

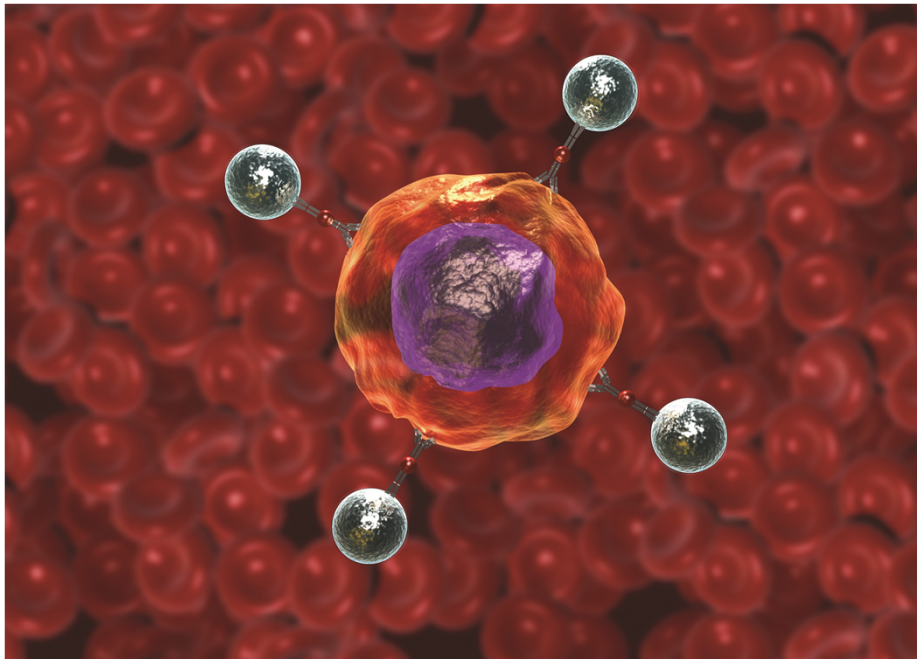
Congestive heart failure NYHA Class IV using MPCs

Acute myocardial infarction using MSCs injected intravenously

Acute myocardial infarction using MPCs delivered by intracoronary catheter

Preclinical

Ischemic stroke using MPCs



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Phase 3 program – NYHA Class II/III congestive heart failure

- MOA - MPCs release multiple factors on interaction with damaged cardiac tissue which induce new blood vessel formation, induce regeneration of heart muscle, reduce scar formation, and have anti-inflammatory properties

- Commercial and development partner Teva is conducting a Phase 3 trial of 1700 patients with New York Heart Association (NYHA) Class II-III chronic congestive heart failure, which includes two interim analyses of efficacy and/or safety

- Double-blinded, 1:1 randomized, placebo-controlled study is evaluating a single dose of 150m MPCs delivered via transendocardial injection catheter to left ventricle of heart failure patients with NYHA Class II or III disease and ejection fraction ≤ 40%

  – Primary efficacy endpoint: time-to-first event analysis of heart failure-related HF- Major Adverse Cardiac Events (MACE), defined as a composite of cardiac-related death or resuscitated cardiac death, or non-fatal decompensated heart failure events
  – Phase 2 trial demonstrated that patients treated with 150m MPC dose did not experience HF-MACE over three-year follow-up period vs. HF-MACE incidence of ~ 30% for controls

- Medical investigators actively recruiting patients

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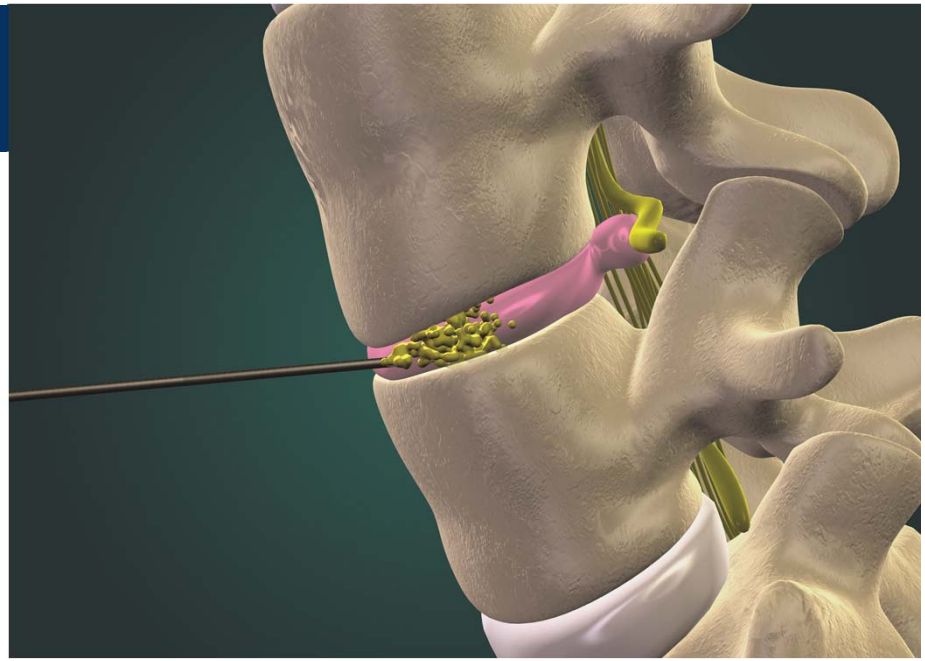
Therapeutic areas of focus

3. Programs in orthopedic conditions of the spine

Phase 3-ready

Degenerative intervertebral disc disease using MPCs (non-surgical)

Lumbar spinal fusion using MPCs (surgical)



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Degenerative disc repair

- Over 4.5 million people in the US have chronic lower back pain due to intervertebral disc disease, no effective treatment other than fusion surgery
- MPCs have been shown in preclinical large animal studies to induce new proteoglycan synthesis, the key substance that is responsible for hydration and maintaining structure of intervertebral discs, and to reverse disc pathology
- Mesoblast is developing non-surgical adult stem cell treatment for patients with early disc degeneration using an intra-disc injection of allogeneic MPCs

Degenerative disc repair (continued)

- Phase 2 trial in patients >6 months discogenic low back pain failing all other therapies

- 100-patient Phase 2 trial randomized, placebo-controlled comparison of saline, HA, HA + 6M MPC, HA + 18M MPCs injected into culprit painful disc

- 12-month outcome results of Phase 2 trial in 100 patients showed that compared to controls both MPC-treated groups had
  - improvement in chronic low back pain (reduction in mean pain score; increased proportion of patients achieving >50% reduction in pain score; increased proportion of patients achieving minimal residual back pain);
  - reduced opioid use for pain relief;
  - reduced need for additional surgical and non-surgical interventions for persistent pain;
  - improvement in function (reduction in mean disability score and increased proportion of patients achieving minimal residual functional disability);
  - radiographically-determined improvement in disc stability

- Phase 3 trials planned for intervertebral disc repair, EOP2 FDA meeting in Q2

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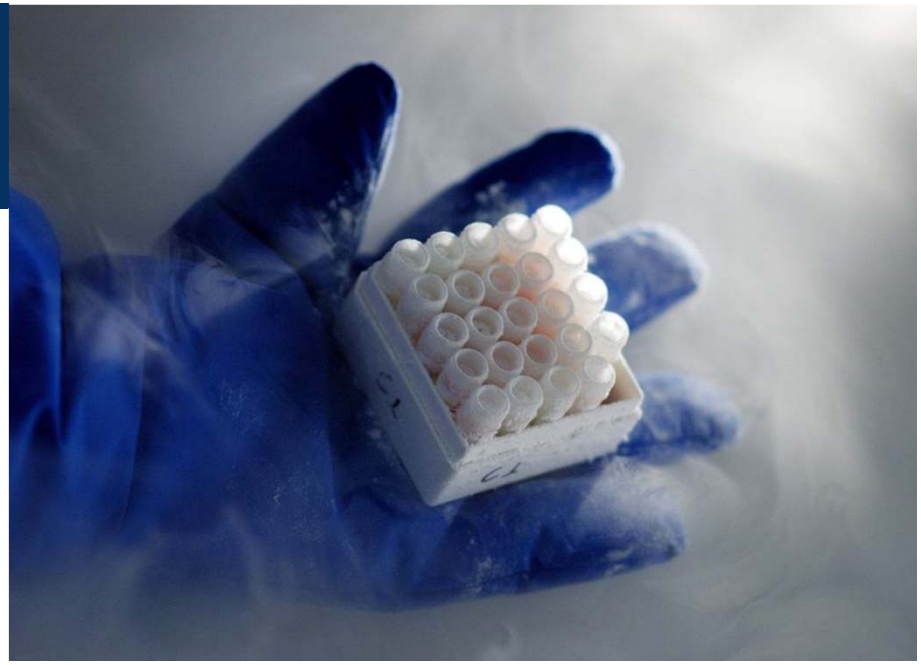
Therapeutic areas of focus

4. Programs in oncology conditions associated with bone marrow transplantation

Commercial Ready
Pediatric GVHD (Canada & New Zealand)
Phase 3
Cord blood expansion for hematological malignancies



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Oncology – acute Graft Versus Host Disease (GVHD)

- ~29,500 allogeneic hematopoietic stem cell transplants (HSCTs) performed annually for treatment of hematologic malignancies, certain forms of anemia, and immunological deficiencies
- GVHD is a potentially life-threatening complication that arises in approximately 50% of all patients who receive an HSCT and affects the skin, gastrointestinal tract, and liver
- Mortality can reach 85% in patients with liver and gut complications

- In Phase 3 trial, Prochymal® significantly improved overall responses in the adult subset with gut or liver GVHD and resulted in improved survival
- In open-label pediatric study, at day 28 61% of patients were responders to Prochymal®
- Response at day 28 to Prochymal® therapy was a significant predictor of improved day 100 survival ($p<0.001$)
- Day 100 survival was 76% in Prochymal® responders, compared to 78% in non-responders (p value <0.001, log rank test)

Conditionally approved for use in children in Canada and New Zealand

- *Planned regulatory discussions regarding accelerated product approvals in major markets*

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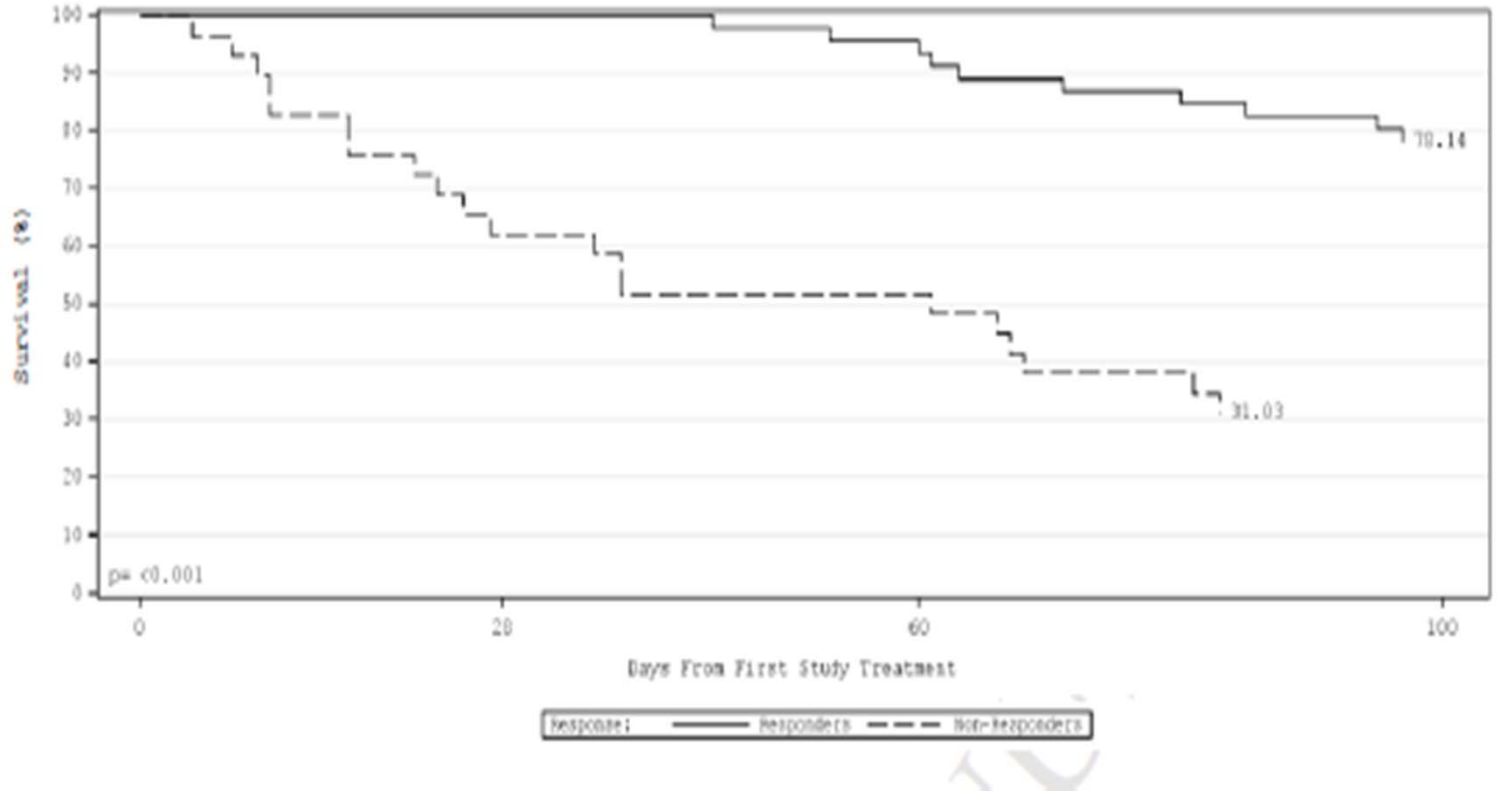
Acute GvHD - Prochymal®

Patients who responded to therapy by day 28 had a higher Kaplan-Meier estimated probability of 100-day survival than patients who did not respond (78% vs. 28%, p<0.001).



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Key deliverables in 2014

- Regulatory submissions in major markets for GvHD product approvals
- Commencement of Phase 3 trial in intervertebral disc disease
- Continued recruitment of Phase 3 trials in congestive heart failure, Crohn's disease, and expanded cord blood for bone marrow transplantation
- Continued recruitment and results from Phase 2 trials of diabetic nephropathy, AMI, RA
- Commercial scale manufacturing and bioprocessing in preparation for commercial launches
- Evolving strategy for early product approvals in Japan

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Leading the world in the development of adult stem cell therapies

26 February 2014