#### **UNITED STATES** SECURITIES AND EXCHANGE COMMISSION Washington, D.C. 20549

Form 6-K

Report of Foreign Private Issuer Pursuant to Rule 13a-16 or 15d-16 under the Securities Exchange Act of 1934

For the month of January 2018

Commission File Number 001-37626

### **Mesoblast Limited**

(Exact name of Registrant as specified in its charter)

Not Applicable (Translation of Registrant's name into English)

Australia (Jurisdiction of incorporation or organization)

Silviu Itescu Chief Executive Officer and Executive Director Level 38 55 Collins Street

Melbourne 3000 Australia (Address of principal executive offices)

Indicate by check mark whether the registrant files or will file annual reports under cover Form 20-F or Form 40-F:

Form 20-F 🛛 Form 40-F 🗆

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(1): Yes 🗆 No 🗹

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(7):

Yes 🗆 No 🗵

### INFORMATION CONTAINED ON THIS REPORT ON FORM 6-K

On January 12, 2018, Mesoblast Limited filed with the Australian Securities Exchange a new investor presentation, which is attached hereto as Exhibit 99.1, and is incorporated herein by reference.

### SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly organized.

MESOBLAST LIMITED

/s/ Charlie Harrison

Charlie Harrison Company Secretary

Dated: January 18, 2018

<u>Item</u> 99.1





# Cellular Medicines for Intractable Serious and Life-Threatening Diseases

# J.P. Morgan 2018 Healthcare Conference

January 2018 Nasdaq: MESO ASX: MSB



### CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This presentation includes forward-looking statements that relate to future events or our future financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements processed or implied by these forward-looking statements. We make such forward-looking statements of historical facts contained in this presentation are forward-looking statements of historical facts contained in this presentation are forward-looking statements. We make such forward-looking statements of historical facts contained in this presentation are forward-looking statements. We thave based these forward-looking statements largely on our current expectations and future events , recent changes in regulatory laws, and financial rends that we believe may affect our out limited to, business strategy and financial needs. These statements may relate to, but are not limited to expectations regarding the safety or efficacy of, or potential applications for, Mesoblast's adult stem cell technologies; expectations regarding the sterety of manufacturing processes; expectations about Mesoblast's ability to grow its business and statements concerning Mesoblast's capital requirements and ability to raise future capital, among others. Forward-looking statements any elider from the realits and ability to raise future capital, among others. Forward-looking statements concerning Mesoblast's capital requirements and ability to raise future capital, among others. Forward-looking statements are verted to the set event and adverse. You should not be read as a guarantee of future performance or activements to be material and currence and initide to we best or on our website. Uncertainties and results may cause expressed or implied by the effort. Forward-looking statements concerning the schlast or concerning the schlast or on our website. Uncertainties and results may cause expenses and the set evelopment and evertex. You are assetted there to a set and the prea

# Our Mission:

Mesoblast is committed to bring to market disruptive cellular medicines to treat serious and life-threatening illnesses

### Investment Proposition:

Building a Leading Franchise of Cellular Medicines

- Disruptive Cellular Technology Platform
- Commercial Translation Capabilities
- Advanced Pipeline of Cellular Medicines
- Targeting Serious or Life-Threatening Conditions with Unmet Needs

### **Disruptive Cellular Medicine Platform<sup>1-4</sup>**

- STRO-1<sup>+</sup> Mesenchymal Precursor Cells (MPCs) are at the apex of the hierarchy of Mesenchymal Lineage cells
- STRO-1/STRO-3 immuno-selection provides a homogeneous population of MPCs with unique receptors that respond to activating inflammation and damaged-tissue signals
- In response to activating signals present in the endogenous environment, MPCs secrete a diverse variety of biomolecules responsible for immunomodulation and tissue repair
- The multi-modal mechanisms of action target multiple pathways
- Simmons PJ and Torok-Storb, B. Identification of stromal cell precursors in bone marrow by a novel monocloncal antibody, STRO-1. Blood. 1991;78:55-62.
- Gronthos S, Zannettino AC, Hay SJ, et al. Molecular and cellular characterisation of highly purified stromal stem cells derived from human bone marrow. J Cell Sci. 2003;116(Pt 9):1827-35.
   See F, Seki T, Psaltis PJ, et al. Therapeutic effects of human STRO-3-selected mesenchymal precursor cells and their soluble factors
- in experimental myocardial ischemia. J Cell Mol Med. 2011;15:2117-29. 4. Psaltis PJ, Paton S, See F, et al. Enrichment for STRO-1 expression enhances the cardiovascular paracrine activity of human bone
- PsaltisPJ, Paton S, See F, et al. Enrichment for STRO-1 expression enhances the cardiovascular paracrine activity of human bone marrow-derived mesenchymal cell populations. J Cell Physiol. 2010;223(2):530-40.



# **Commercial Translation Capabilities:** Technology Positioned for Scalable, Industrialized Manufacturing

- Immune privileged nature of STRO-1+ MPCs enables allogeneic "off the shelf" product candidates
- Culture expansion scalable to produce commercial quantities of potent and reproducible therapeutic doses
- In-house proprietary media formulations and commercial-grade bioreactors to deliver step-change yield improvements
- Specific formulations defined for product delineation
- Management know how in regulatory activities necessary for product approval and commercial launch
- TEMCELL® HS. Inj., first allogeneic cellular medicine received full approval in Japan and successfully launched for acute Graft vs Host Disease<sup>1</sup>



Lonza contract manufacturing facility in Singapore

<sup>1.</sup> TEMCELL®HS. Inj. Is the registered trademark of JCR Pharmaceuticals Co. Ltd., Mesoblast's Licensee.

### **Portfolio of Advanced Product Candidates:**

Three Tier 1 Product Candidates in Phase 3

Platform	Product Candidate	Therapeutic Area	Pre-Clinical/ Pre-IND	Phase 2	Phase 3	Approval	Partnering <sup>1</sup>
MPC MPC MPC MSC	MPC-150-IM MPC-06-ID MPC-300-IV TEMCELL® HS Inj MSC-100-IV	Advanced (Class 3) HF End Stage (Class 4) HF1 Chronic Low Back Pain RA DN/Type 2 Diabetes Acute GVHD Acute GVHD		_	_	Japan	<ul> <li>Imesoblast the regenerative medicine company</li> <li>Imesoblast the regenerative medicine company</li> <li>Imesoblast the regenerative medicine company</li> <li>Imesoblast the regenerative medicine company</li> </ul>
Includes MSC-100-IV (Crohn's disease – biologic refractory), MPC-25-IC (Acute Cardiac Ischemia), MPC-25-Osteo (Spinal Fusion) and MPC-75-IA (Knee Osteoarthritis) This chart is figurative and does not purport to show individual trial progress within a clinical program. For product registration purposes, Phase 3 programs may require more than one trial. Tier 1 programs represent our lead programs where we focus the majority of our time and resources. Tier 2 programs are also in development and may advance to Tier 1 depending on the merit of newly generated data, market opportunity or partnering options.							

### The 21<sup>st</sup> Century Cures Act ("Cures Act"):

Legislation for An Expedited Approval Path for Cellular Medicines Designated as Regenerative Medicine Advanced Therapies (RMAT)

- Cellular medicines may be designated as regenerative advanced therapies, if they are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition, and there is preliminary clinical evidence indicating the potential to address the unmet medical need
- Key benefits of the legislation for cell-based medicines, designated as regenerative advanced therapies, include:
  - Potential eligibility for priority review and accelerated approval
  - Potential to utilize surrogate endpoints for accelerated approval
  - Potential to utilize a patient registry data and other sources of "real world evidence" for post approval studies, subject to approval by the FDA

Our Portfolio of Advanced Product Candidates is Well Positioned to Achieve Accelerated Approvals Under the Cures Act

### December 2017 Mesoblast Received FDA RMAT Designation For Its Cell Therapy In Heart Failure Patients With Left Ventricular Assist Devices (LVADs)

- RMAT designation grant was based on the completed study data set and related analyses of a 30-patient randomized, blinded, placebo controlled trial in end-stage heart failure patients with LVADs which suggested:
  - Improved native heart function
  - Prolonged the time post LVAD implantation of a first hospitalization for a non-surgical GI bleeding event
  - Improved early survival rates
- 159 patient trial in end-stage heart failure with LVADs has completed enrollment and the primary endpoint will be reached in Q1 CY 2018
- Mesoblast intends to have a multidisciplinary comprehensive discussion as soon as possible with the FDA
  regarding the development strategy and evidence needed to support approval of its allogeneic MPC
  product candidate for end-stage heart failure patients with LVAD in an efficient manner

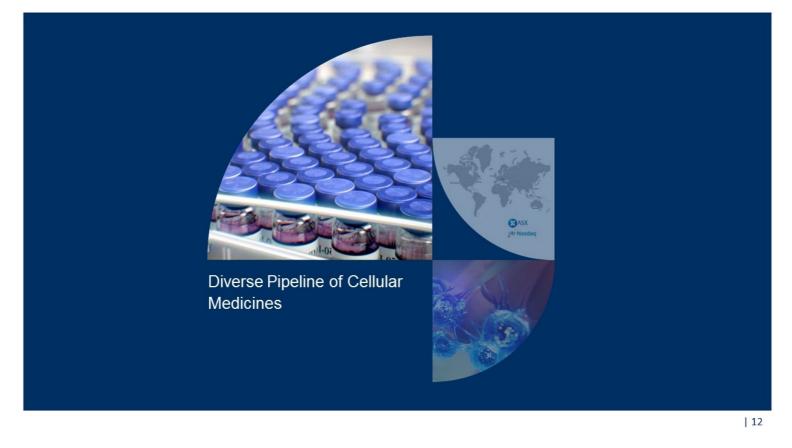
- Composition of Matter, Manufacturing, and Therapeutic Applications of Potent Immuno-selected mesenchymal lineage precursor and stem cells
- 800 Patents and patent applications across 69 Patent Families. Protection across major markets including the U.S., Europe, Japan and China



# December 2017 Mesoblast Concluded Patent Settlement and License Agreement With TiGenix

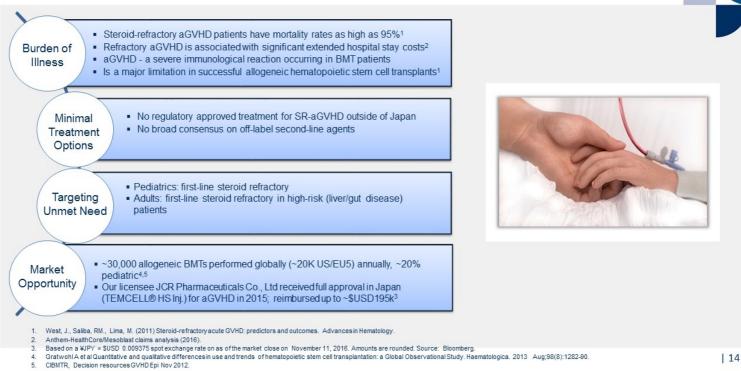


- Mesoblast granted TiGenix exclusive access to certain of its patents to support global commercialization
  of the adipose-derived mesenchymal stem cell (MSC) product Cx601 limited to the local treatment of
  fistulae, including in Crohn's disease
- Mesoblast continues to develop its proprietary bone marrow-derived allogeneic expanded MSC product candidate for intravenous delivery to induce remission in patients with biologic-refractory Crohn's disease
- Mesoblast will receive up to €20 million in payments (approx. US\$24 million), with €5 million upfront,
   €5 million within 12 months and up to €10 million in product regulatory milestones
- Mesoblast will additionally receive single digit royalties on global net sales of Cx601 for fistulae
- Subsequent to the patent settlement and license agreement, Takeda announced its intention to build upon its prior exclusive ex-USA license for Cx601 from TiGenix by acquiring TiGenix for approximately €520 million



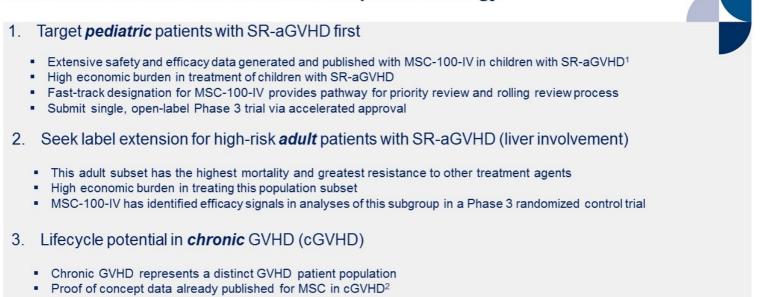
# Acute Graft vs Host Disease (aGVHD) MSC-100-IV for Steroid-Refractory aGVHD

### MSC-100-IV: Market Opportunity for aGVHD



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### MSC-100-IV for aGVHD: Product Development Strategy



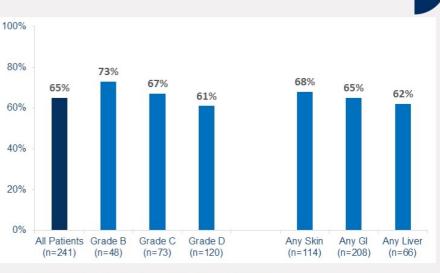
1. Allogeneic Human Mesenchymal Stem Cell Therapy (Remestemcel-L, Prochymal) as a Rescue Agent for Severe Refractory Acute Graft-versus-Host Disease in Pediatric Patients - Biology of Blood and Marrow Transplantation Journal, August 2013. 2. Khandelwal P, Teusink-Cross A, Davies S (2017) Ruxolitinib as Salvage Therapy in Steroid-Refractory Acute Graft-versus-Host Disease in Pediatric Hematopoietic Stem Cell Transplant Patients. Biol Blood Marrow Transplant 23; 1122-1127 2. Weng JY, Du X, Geng SX, Peng YW, Wang Z, Lu ZS et al. Mesenchymal stem cell as salvage treatment for refractory chronic GVHD. Bone Marrow Transplant 45: 1732-1740 (2010)

### **MSC-100-IV: Expanded Access Program**

Overall Day 28 Response in Pediatric aGVHD Patients Receiving MSC-100-IV as First-line or Salvage Therapy After Failing Steroids

# Population: steroid-refractory aGVHD pediatric patients

- 241 pediatric patients undergoing HSCT were enrolled and treated at 50 sites in North America and Europe from 2007-2014
- Ages 2 months 17 years
- Acute GvHD grades B-D (CIBMTR)
- Failed steroid treatment and multiple other agents
- aGVHD not improving after at least 3 days of methylprednisolone (at least 1 mg/kg/day or equivalent)



Complete Response was 14%, Partial Response was 51%

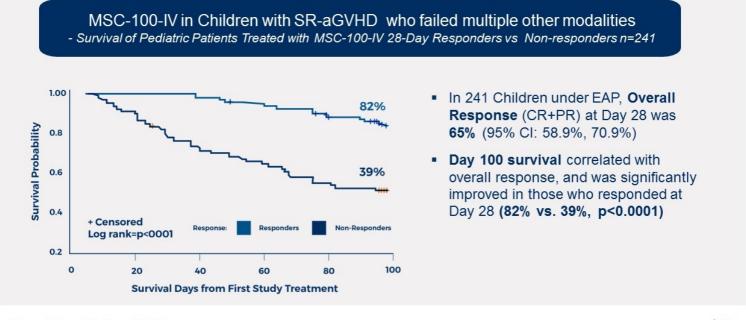
 Responses were observed for all GVHD grades and did not differ by baseline organ involvement

Kurtzberg et al: Presentation Tandem Feb 2016

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### **MSC-100-IV: Expanded Access Program**

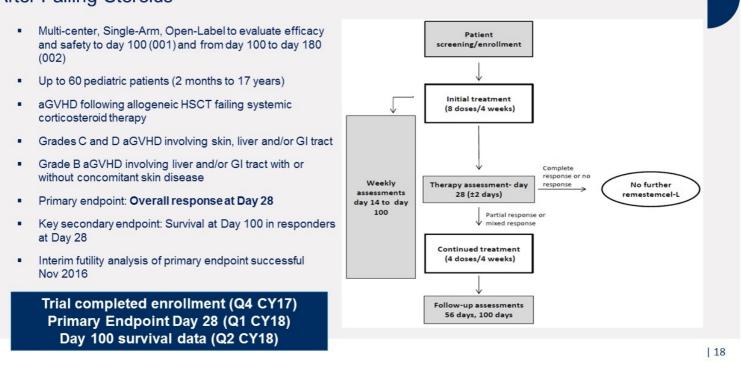
Correlation of Day 28 Overall Response with Day 100 Survival, Using MSC-100-IV as First-line or Salvage Therapy After Failing Steroids and/or Additional Treatments



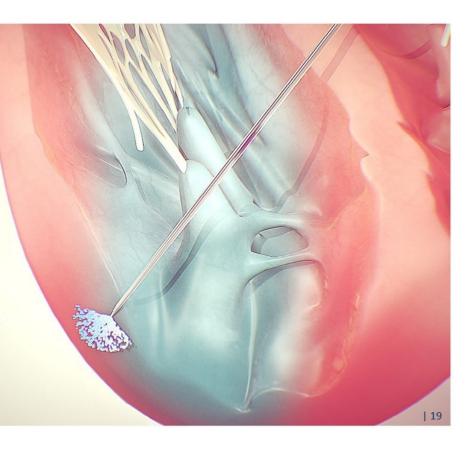
Kurtzberg et al: Presentation Tandem Feb 2016

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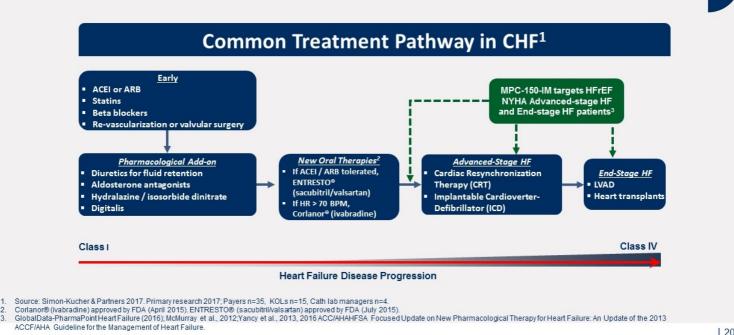
### **MSC-100-IV:** Phase 3 Pediatric Trial Completed Enrollment as First-line Therapy in aGVHD After Failing Steroids







### **MPC-150-IM:** Targeting Patients with Worsening HF Despite Optimal Standard of Care

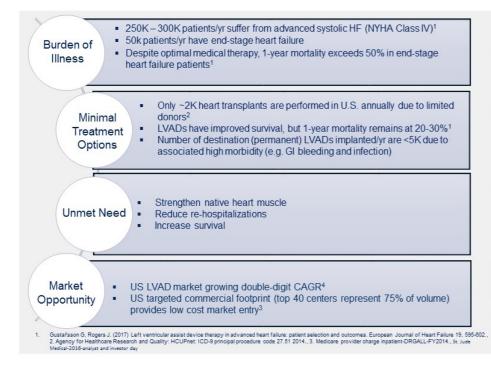


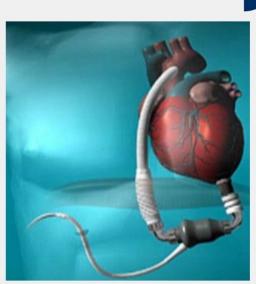
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### MPC-150-IM: Product Development Strategy Following RMAT Designation for Heart Failure Patients With Left Ventricular Assist Devices (LVADs)

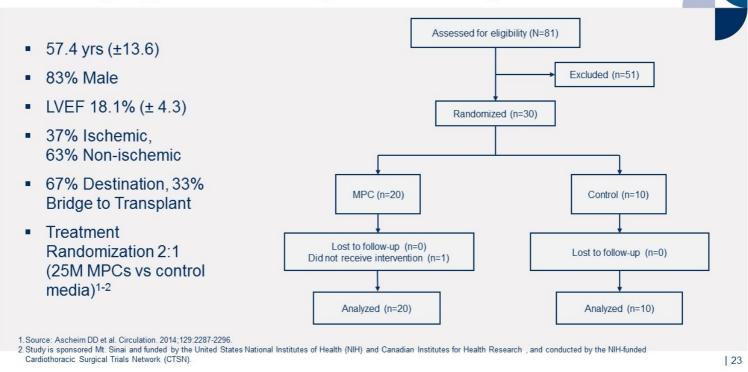
- 1. Leverage data for potential near term market entry opportunity for MPC-150-IM in end-stage heart failure patients with LVADs
- 1. Broaden market potential by creating Bridge to Recovery (BTR) market, representing a high-growth market opportunity for temporary LVAD use and explantation in end-stage, Class-IV heart failure patients
- 1. Label extension through completion of phase 3 program (DREAM-HF) in NYHA class IIb/III heart failure patients

## MPC-150-IM: Class IV Market Opportunity





### MPC-150-IM Proof of Concept Randomized Controlled Phase 2 Trial Which Successfully Supported RMAT Designation: Trial Design



### MPC-150-IM Proof of Concept Randomized Controlled Phase 2 Trial Which Successfully Supported RMAT Designation: Trial Results



- Median time to first hospitalization was 91 days in the MPC group vs 51 days in the control group
- 50% of MPC vs. 20% of control patients tolerated temporary wean at 90 days despite low dose of cells deployed
- Total number of temporary weans tolerated by MPC group was more than double that of the control group
- Using Bayesian approach, posterior probability that MPCs increased likelihood of successful wean at 90 days was 93%
- At 90 days, 30% (3/10) of controls expired compared to 0% (0/20) treated patients

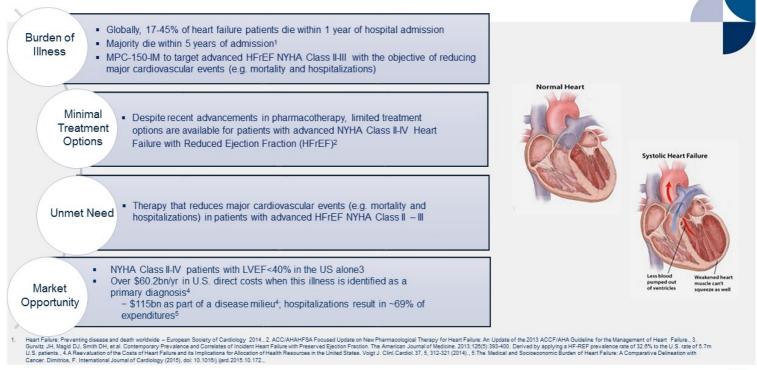
1. Source: Ascheim DD et al. Circulation. 2014;129:2287-2296.

### MPC-150-IM:

Phase 2b Trial Evaluating 150M MPCs in End-Stage Heart Failure Patients with LVADs

- The 159-patient, double-blind, placebo-controlled 2:1 randomized trial, is evaluating the safety and efficacy of injecting MPC-150-IM into the native myocardium of LVAD recipients
- Enrollment completed in Q3, CY2017
- Key safety and efficacy endpoints of the study:
  - Number of temporary weans from LVAD tolerated
  - Time to re-hospitalization
  - Patient survival
  - Various quality of life measurements
- Study is sponsored by Icahn School of Medicine, funded by the United States National Institutes of Health (NIH) and Canadian Health of Research Institute, and conducted by the NIH-funded Cardiothoracic Surgical Trials Network (CTSN)
  - RMAT designation for end stage heart failure with LVADs granted December 2017
  - End stage heart failure trial six-month primary endpoint Q1 CY18
  - End stage heart failure trial full data read-out Q3 CY18

### MPC-150-IM: Class III Heart Failure Market Opportunity

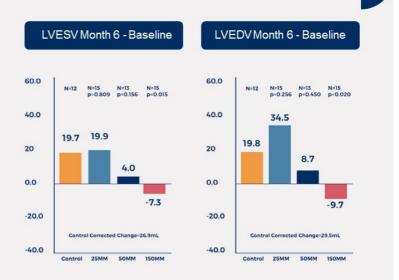


## **MPC-150-IM:**

Phase 2 Randomized Placebo Controlled Trial in 60 Patients HF Class II/III and LVEF<40%

### Objectives

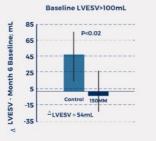
- Identify a dose response and an optimal therapeutic dose
- Identify optimal target population for therapeutic effect
- Placebo vs. 25, 75, 150M MPCs injected by endomyocardial catheter
- At 6 months: Dose-dependent effect seen on left ventricular remodeling, with 150M cell dose (MPC-150-IM) showing greatest effect vs. controls

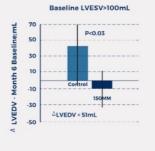


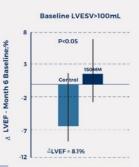
Source: Circ Res. 2015; 117:576-584. Perin E et al. A Phase II Dose-Escalation Study of Allogeneic Mesenchymal Precursor Cells in Patients With Ischemic or Non-Ischemic Heart Failure.

### **MPC-150-IM:** Therapeutic Benefit on LV Remodeling Enhanced in Phase 2 Subjects with LVESV >100ml

 Placebo corrected benefit of 150M cell dose on cardiac volumes and ejection fraction at 6 months was greatest in patients with more advanced heart failure as defined by baseline LVESV>100ml at baseline







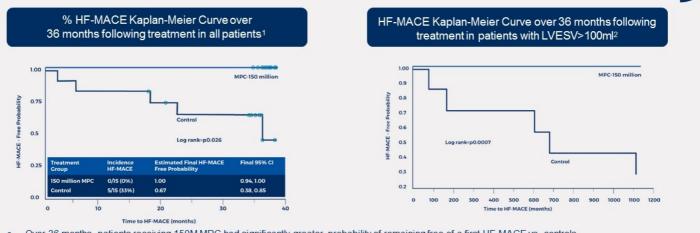
		Change (Entire cohort) Month 6 minus baseline			Change (LVES			
		PBO (n=15)	150M MPC (n=15)	∆, PBO corrected	PBO (n=7)	150M MPC (n=11)	∆, PBO corrected	P-values
	LVESV	+20	-7	-27	+46	-8	-54	<0.02
	LVEDV	+20	-10	-30	+41	-10	-51	<0.03
	LVEF	-2.3	+0.6	+2.9	-6.4	+1.7	+8.1	<0.05

Source : Perin et al., Journal of Cardiac Failure 2015; Vol 21(8): S107; 19th Annual Scientific Meeting of the Heart Failure Society of America, Emerson et al.

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### MPC-150-IM:

Durable (36 Months) Protection Against HF-MACE<sup>1</sup> in Phase 2 Trial Following Single Dose in NYHA Class II/III With Reduced Ejection Fraction



- Over 36 months, patients receiving 150M MPC had significantly greater probability of remaining free of a first HF-MACE vs. controls (0% vs. 33%, p = 0.026 by log-rank)
- All HF-MACE events occurred in controls with baseline Left Ventricular End Systolic Volume (LVESV)>100ml, where the treatment effect size was even greater (0% vs. 71%, p = 0.0007 by log rank)
- Controls with baseline LVESV>100ml had 11 total/recurrent HF-MACE events over 36 months vs. 0 in matched patients receiving 150M MPCs (p=0.0007)

1. HF-MACE is defined as a composite of cardiac related death or non-fatal heart failure hospitalisations. 2. Circ Res. 2015; 117:576-584. Perin E et al. A Phase II Dose-Escalation Study of Allogeneic Mesenchymal Precursor Cells in Patients With Ischemic or Non-Ischemic Heart Failure 3. Journal of Cardiac Failure 2015; Vol 21(8): S107; 19th Annual Scientific Meeting of the Heart Failure Society of America, Emerson et al.

### MPC-150-IM: Phase 3 Trial Targets Advanced Heart Failure

NYHA class II/III patients with large baseline LVESV and advanced heart failure are at highest risk of heart failure-related major adverse cardiac events (HF-MACE)

- Have increased likelihood of having recurrent HF hospitalizations
- Existing therapies are limited and economic burden is greatest

### The ongoing Phase 3 trial is enriched for HF patients with high risk of HF-MACE

- Enrichment for these patients based on heart failure hospitalization in the past 9 months and/or significantly elevated baseline NT-proBNP
- Primary endpoint is a comparison of recurrent non-fatal HF-MACE between cell-treated NYHA class II/III patients and controls
- Terminal events (such as death, implantation of a mechanical heart assist device or a heart transplant) are also being analyzed as they relate to non- fatal recurrent HF-MACE

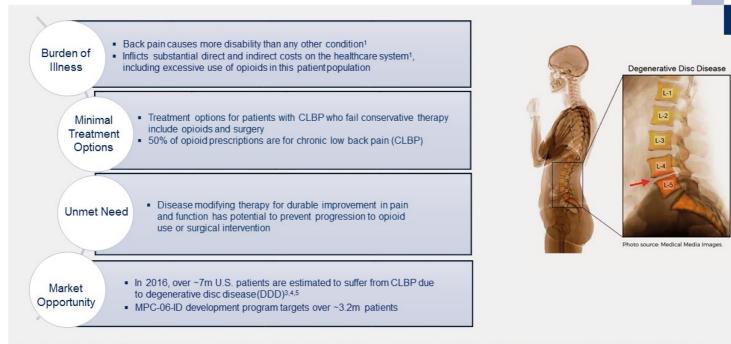
## MPC-150-IM:

Operational Update for Phase 3 Trial in NYHA Class II-III Advanced CHF Patients

- Trial has enrolled more than 400 of approximately 600 patients
- In April 2017, a pre-specified interim futility analysis of the efficacy endpoint in the Phase 3 trial's first 270 patients was successfully achieved
- After completing the interim analysis, the trial's Independent Data Monitoring Committee (IDMC) formally recommended the trial be continued as planned
- Phase 3 trial targeted enrollment completion (2H CY18)



# **MPC-06-ID:** A Non-Opioid Alternative for Chronic Low Back Pain Due to Degenerative Disc Disease



Williams, J., NG, Nawi, Pelzter, K. (2015) Risk factors and disability associated with low back pain in older adults in low-and middle-income countries. Results from the WHO Study on global ageing and adult health (SAGE). PloS One. 2015; 10(6): e0127880, 2: Simon, J., McAullife, M., Shamim, F. (2015) Discogenic Low Back Pain. Phys Med Rehabil Clin N Am 25 (2014) 305–317., 3. Decision Resources: Chronic Pain December 2015., 4. LEK & NCI opinion leader interviews, and secondary analysis., 5. Navigant: Commercial Assessment for a Proprietary Cell-Based Therapy for DDD in the U.S. and the EU3 – August 2014., 6. HealthCare Utilization and Cost of Discogenic Lower Back Pain in the US – Anthem/HealthCore.,

## **The Opioid Epidemic**

- 50% of opioid prescriptions are for chronic low back pain (CLBP)
- Over 1,000 people are treated in U.S. emergency departments everyday for misusing prescription opioids
- Over 33,000 people in the U.S. died of prescription opioid related overdoses in 2016
- Opioid epidemic declared a public health emergency by U.S. President Trump in October, 2017
- A non-opioid solution for CLBP is imperative

The 21<sup>st</sup> Century Cures Act includes measures to combat opioid dependence and accelerated approval for non-opioid pain reducing drugs

Information derived from Centers for Disease Control and Prevention, National Center for Health Statistics. Underlying Cause of Death 1999-2015 on CDC WONDER Online Database, released December, 2016. Available at: <u>http://wonder.cdc.gov/ucdicd10.htm</u>]. Substance Abuse and Mental Health Services Administration. Key Substance Use and Mental Health Indicators in the United States: Results from the 2015 National Survey on Drug Use and Health. Online Database, released September, 2016. Available at: <u>https://www.samhsa.gov/data/sites/default/files/NSDUH-FFR1-2015/NSDUH-FFR1-2015/NSDUH-FFR1-2015/NSDUH-FFR1-2015/NSDUH-FFR1-2015/NSDUH-FFR1-2015/NSDUH-FFR1-2015/NSDUH-FFR1-2015.htm Jones CM. Heroin use and heroin use risk behaviors among nonmedical users of prescription opioid pain relievers - United States, 2002-2004 and 2008-2010. Drug Alcohol Depend. 2013 Sep 1;132(1-2):95-100. doi: 10.1016/j.drugalcdep.2013.01.007.Epub 2013 Feb 12.</u>

## MPC-06-ID: Phase 2 Trial Results Support Phase 3 Program

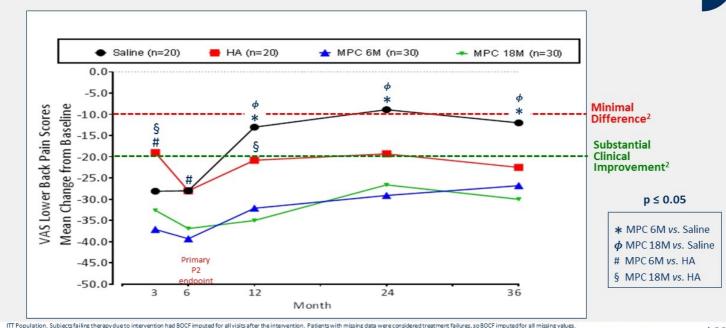
- 100 patients with >6 months of CLBP due to DDD and unresponsive to conservative therapies (incl. opioids and epidural steroids) were evaluated in a blinded, randomized, placebo controlled Phase 2 trial
- Primary endpoint composite over 24 months was achieved by 41% of patients who received 6 million MPCs, 35% of the 18 million MPC group, 18% of the hyaluronic acid group, and 13% of the saline group, using the pre-specified PP population



1. Source Mesoblast Ltd; PP = Per Protocol population. A Composite Responder must have an optimal pain (50% reduction in VAS) AND function (15 point reduction in ODI) response AND no additional intervention.

## MPC-06-ID: Phase 2 Clinical Trial Results:

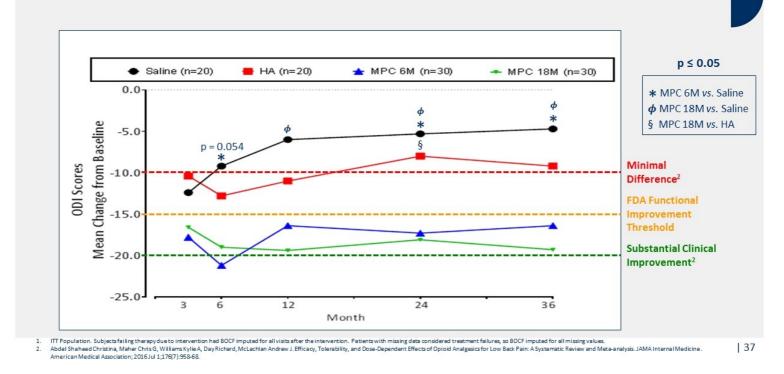
Substantial Reduction in Low Back Pain Through 36 Months After Single Dose



ITT Population. Subjects failing therapydue to intervention had BOCF imputed for all visits after the intervention. Patients with missing data were considered treatment failures, so BOCF imputed for all missing values.
 Abdel Shaheed Christina, Maher Chris G, Williams Kylie A, Day Richard, McLachlan Andrew J. Efficacy, Tolerability, and Dose-Dependent Effects of Opioid Analgesics for Low Back Pain: A Systematic Review and Meta-analysis. JAMA Internal Medicine.
 American Medical Association; 2016 Jul 1;176(7):958-68.

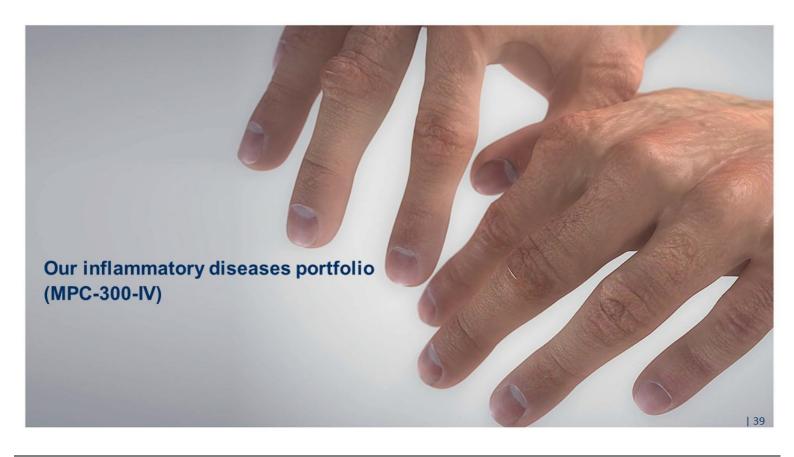
## MPC-06-ID: Phase 2 Clinical Trial Results

Reduction in Functional Disability Through 36 Months After Single Dose



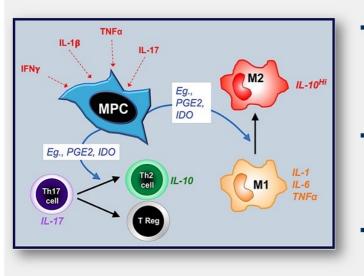
- A 360-patient Phase 3 trial across U.S. and Australian sites
- Targeted to complete recruitment early Q1 CY18
- FDA has provided written guidance:
  - Use of a composite primary endpoint at 12 and 24 months is acceptable
  - Agreed thresholds for pain (50% decrease in VAS) and function (15 point improvement in ODI)
  - No additional intervention at the treated level through 24 months

If the P3 results replicate P2 results in pain and function, leverage this product candidate as a potential non-opioid treatment option for chronic low back pain



## MPC-300-IV:

Being evaluated in immune mediated diseases where the cellular product candidate responds to multiple inflammatory signals by releasing factors that modulate the immune response



#### Phase 2 Clinical Data in Immune Mediated Diseases

- 60 patients, type 2 diabetes with inadequately controlled glucose:
  - Randomized, placebo controlled dose-ranging study completed
  - Positive dose-dependent effects seen on reduction in HbA1c at 3 months<sup>1</sup>

#### 30 patients, diabetic kidney disease:

- Randomized, placebo controlled dose-ranging study completed
- Positive effects seen on glomerular filtration rate and on inflammatory biomarkers over 6 months<sup>2</sup>

## 48 patients, biologic-refractory rheumatoid arthritis:

 Randomized, placebo controlled, dose-ranging study over 52 weeks

Allogeneic Mesenchymal Precursor Cells in Type 2 Diabetes: A Randomized, Placebo-Controlled, Dose-Escalation Safety and Tolerability Pilot Study - Diabetes Care, July 2015
 Allogeneic Mesenchymal Precursor Cells (MPC) in Diabetic Nephropathy: A Randomized, Placebo-controlled, Dose Escalation Study - E BioMedicine, October 2016

## MPC-300-IV:

Phase 2 trial in biologic refractory Rheumatoid Arthritis shows early and durable effects after single dose

- Infusions were well-tolerated and there were no treatment-related serious adverse events reported, with the safety profile comparable among the placebo and two MPC treatment groups.
- A single intravenous MPC infusion in biologic refractory RA patients resulted in dose-related improvements in clinical symptoms, function, disease activity and patient-reported outcomes. Efficacy signals were observed for each of ACR 20/50/70, ACR-N, HAQ-DI, SF-36 and DAS-28 disease activity score.
- 2 million MPC/kg dose showed greatest overall treatment responses. Onset of treatment response occurred as early as 4 weeks, peaked at 12 weeks, was sustained through 39 weeks, and waned by 52 weeks.
- Greatest benefits over 52 weeks were seen in patients who had failed less than 3 biologics (1-2 biologic sub-group) prior to MPC treatment, identifying this as a potentially optimal target population
  - Phase 2 trial clinical responses along with the safety profile position MPC-300-IV as an early treatment option in RA patients who are resistant or intolerant to anti-TNF or other biologics
  - Future studies will evaluate whether higher doses can induce even greater rates of low disease activity or remission within 12 weeks



## **Targeted Upcoming Milestones and Catalysts**

#### MSC-100-IV for Pediatric Acute GVHD

- Day 28 primary endpoint data read-out (Q1 CY18)
- Day 100 survival data (Q2 CY18)

#### MPC-150-IM for Advanced and End-Stage Heart Failure

- Phase 2B Class IV trial six-month primary endpoint reached (Q1 CY18)<sup>1</sup>
- Phase 2B Class IV trial full data read-out (Q3 CY18)<sup>1</sup>
- Phase 3 trial for Class II/III targeted enrollment completion (H2 CY18)

#### MPC-06-ID for Chronic Low Back Pain

- Phase 3 trial expected to complete enrollment (Q1 CY18)
- Potential Corporate Partnerships

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