mesoblast
the regenerative medicine company

# Global Leader in Allogeneic Cellular Medicines for Inflammatory Diseases

## Financial and Operational Highlights for the Third Quarter Ended March 31, 2020

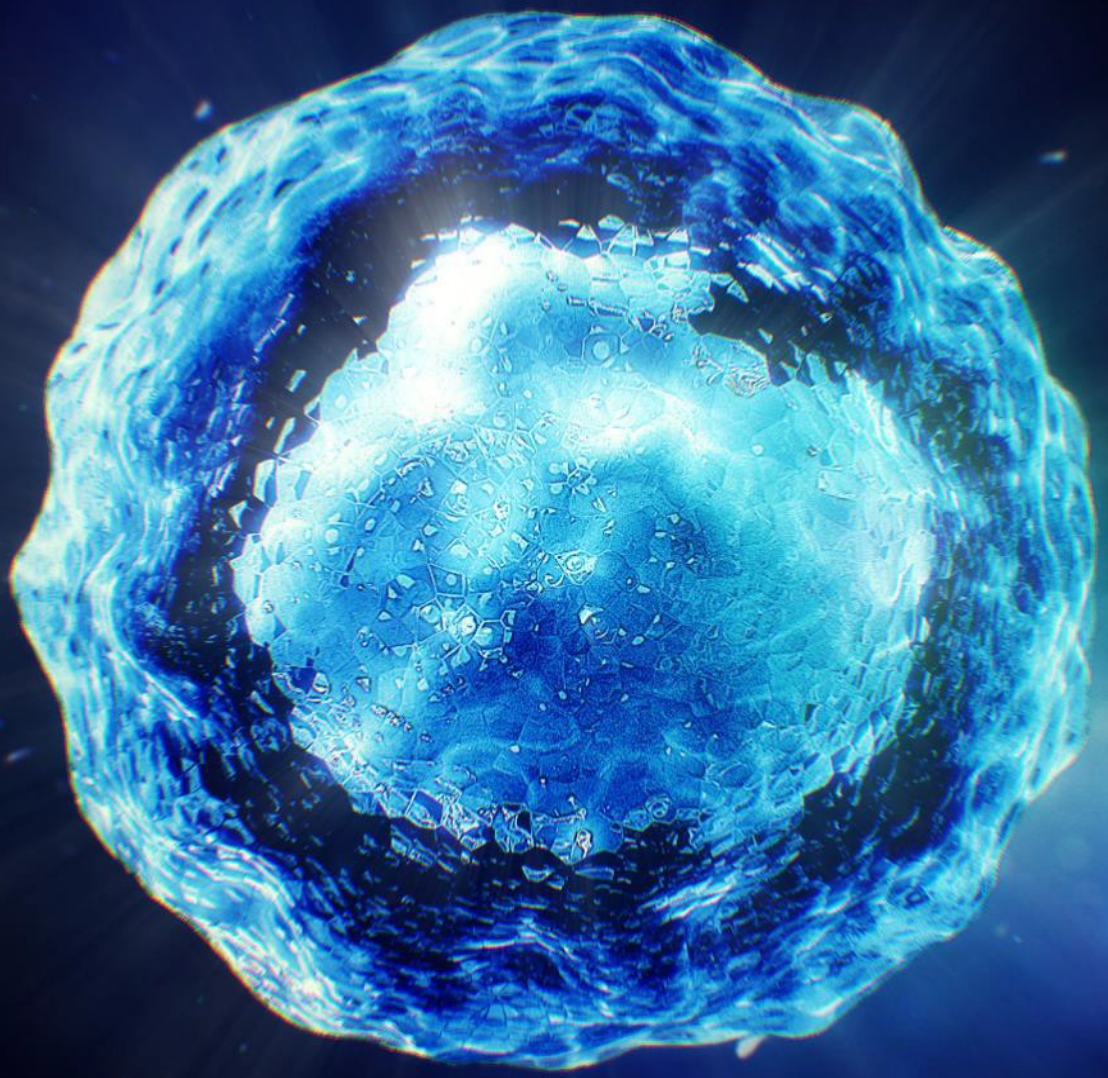
**ASX: MSB; Nasdaq: MESO**

## 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 to differ materially from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this presentation are forward-looking statements. Words such as, but not limited to, "believe," "expect," "anticipate," "estimate," "intend," "plan," "targets," "likely," "will," "would," "could," and similar expressions or phrases identify forward-looking statements. We have based these forward-looking statements largely on our current expectations and future events , recent changes in regulatory laws, and financial trends that we believe may affect our financial condition, results of operation, 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 strength of Mesoblast's intellectual property, the timeline for Mesoblast's regulatory approval process, and the scalability and efficiency of manufacturing processes; expectations about Mesoblast's ability to grow its business and statements regarding its relationships with current and potential future business partners and future benefits of those relationships; statements concerning Mesoblast's share price or potential market capitalization; and statements concerning Mesoblast's capital requirements and ability to raise future capital, among others. Forward-looking statements should not be read as a guarantee of future performance or results, and actual results may differ from the results anticipated in these forward-looking statements, and the differences may be material and adverse. You should read this presentation together with our financial statements and the notes related thereto, as well as the risk factors, in our most recently filed reports with the SEC or on our website. Uncertainties and risks that may cause Mesoblast's actual results, performance or achievements to be materially different from those which may be expressed or implied by such statements, include, without limitation: risks inherent in the development and commercialization of potential products; uncertainty of clinical trial results or regulatory approvals or clearances; government regulation; the need for future capital; dependence upon collaborators; and protection of our intellectual property rights, among others. Accordingly, you should not place undue reliance on these forward-looking statements. We do not undertake any obligations to publicly update or revise any forward-looking statements, whether as a result of new information, future developments or otherwise.*

# Our Mission

Mesoblast is committed to bringing to market innovative cellular medicines to treat serious and life-threatening illnesses



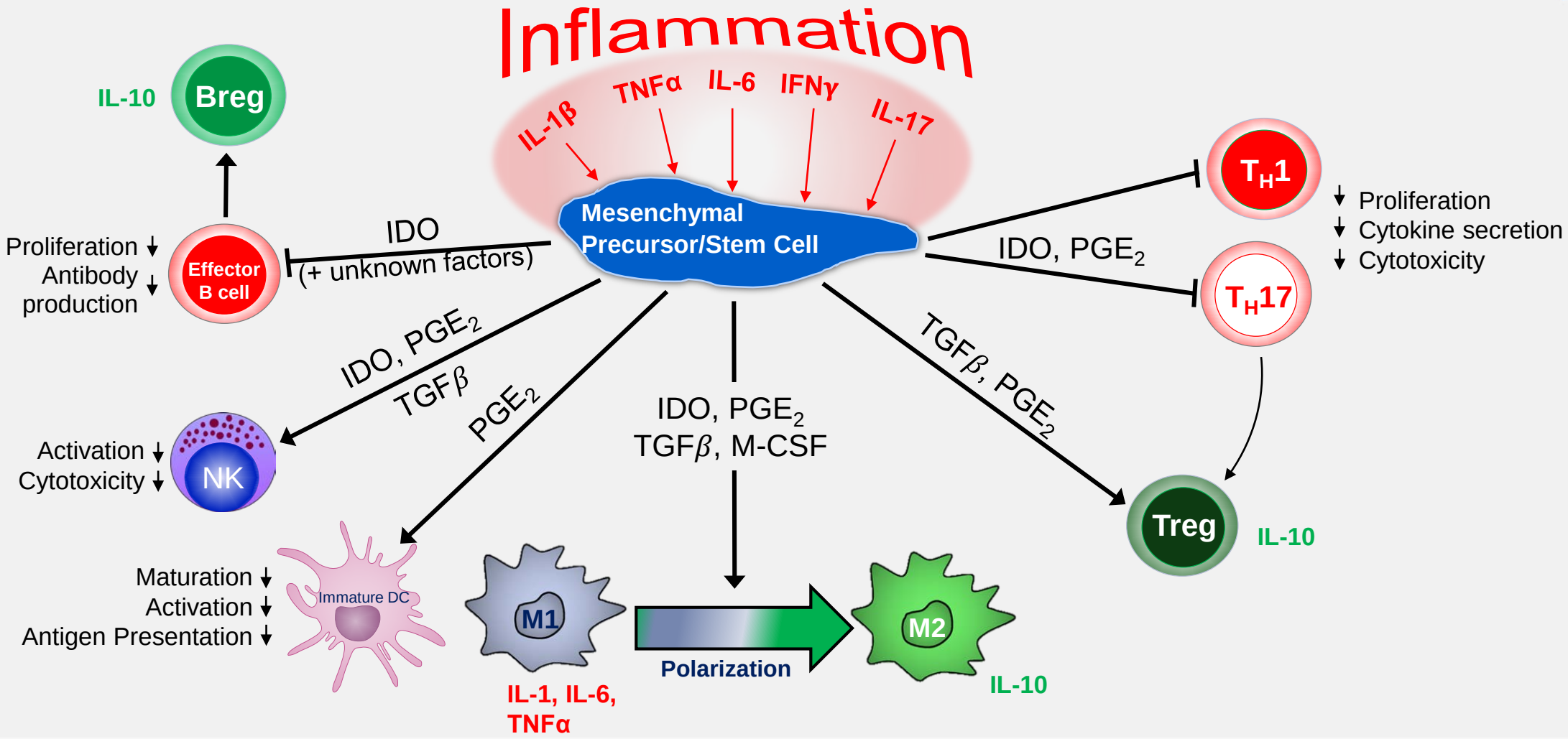
# Allogeneic Cellular Medicines for Inflammatory Diseases

| Innovative Technology Platform | Lead Product Candidate | Pipeline of Phase 3 Product Candidates |
|---|---|---|
| ▪ Allogeneic mesenchymal precursor cells (MPCs) and their progeny, mesenchymal stem cells (MSCs)<br>▪ Well characterized immunomodulatory mechanisms of action<br>▪ Targeting severe and life threatening inflammatory conditions<br>▪ Underpinned by extensive, global intellectual property estate | ▪ RYONCIL™ (remestemcel-L) BLA filed with US FDA for pediatric steroid-refractory acute GVHD<br>▪ Targeted US commercial team for potential launch<br>▪ If approved, launch planned for 2020<br>▪ Industrial-scale manufacturing in place to meet commercial demand<br>▪ Continued growth in royalty revenues from Japan sales of licensee product for acute GVHD[1] | ▪ Lifecycle expansion of remestemcel-L for pediatric and adult inflammatory diseases<br>▪ Phase 3 trial of 300 patients using remestemcel-L in acute respiratory distress syndrome (ARDS) due to COVID-19<br>▪ Two additional product candidates in Phase 3 trials, heart failure and back pain, with near-term US readouts |

1. Licensee JCR Pharmaceuticals Co., Ltd. received the first full PMDA approval for an allogeneic cellular medicine in Japan and markets this product under its trademark, TEMCELL® Hs Inj.

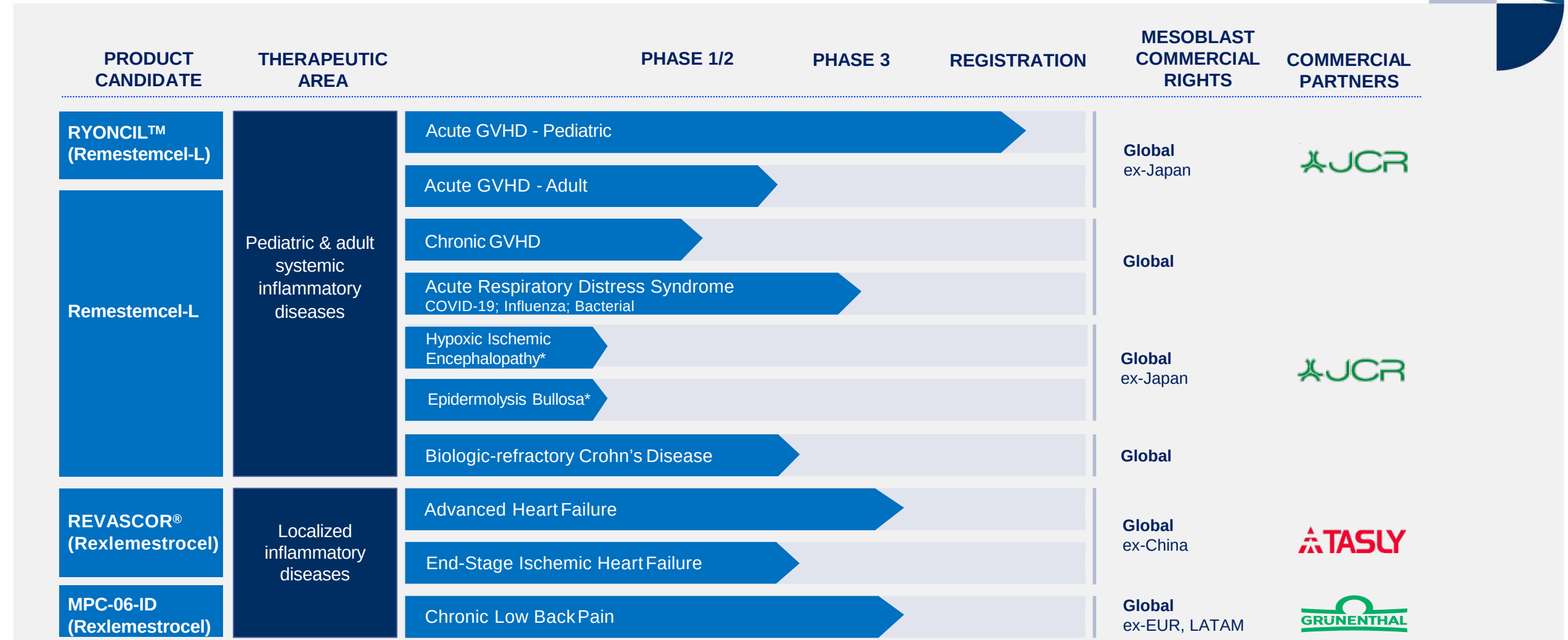
# Platform Technology – Mechanism of Action

*Our cellular therapies are activated by multiple inflammatory cytokines through surface receptors, resulting in orchestration of an anti-inflammatory cascade*




Source: data on file

# Pipeline of Phase 3 Product Candidates




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

* Mesoblast has the right to use data generated by JCR Pharmaceuticals Co Ltd in Japan to support its development and commercialization plans for remestemcel-L in the US and other major healthcare markets, including for GVHD, HIE and EB

# Commercial Scale Manufacturing Capability

- Scalable allogeneic “off-the-shelf” cellular platforms
- Manufacturing meets stringent criteria of international regulatory agencies
- Robust quality assurance processes ensure final product with batch-to-batch consistency and reproducibility
- Current capacity sufficient for RYONCIL GVHD launch

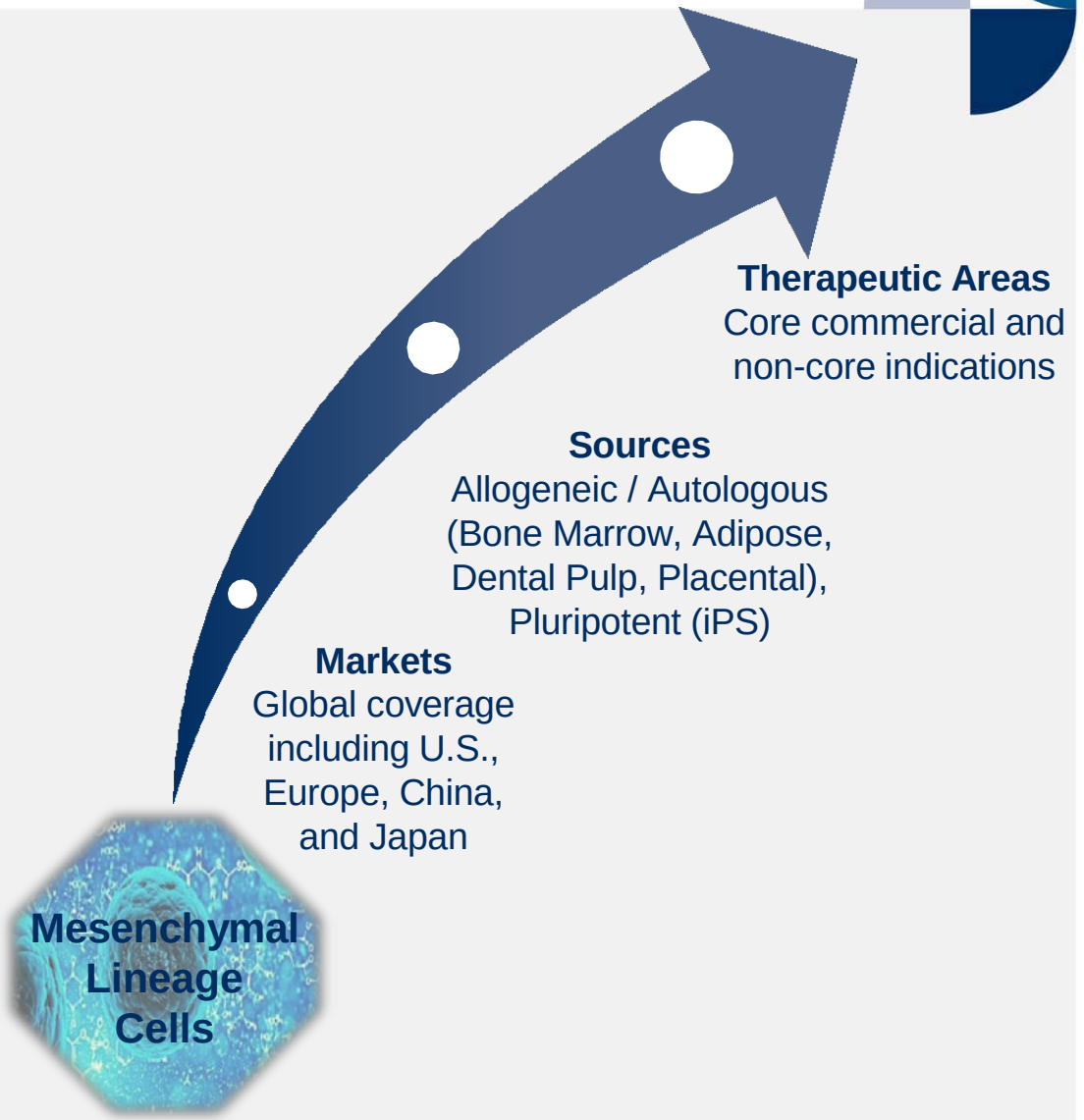
*Manufacturing Remestemcel-L*



- Projected increase in capacity requirements for maturing pipeline, including GVHD label extensions and COVID-19 ARDS
  - Proprietary xeno-free technologies will increase yields and output
  - Moving to 3D bioreactors will reduce labor and improve manufacturing efficiencies
  - These innovations will significantly reduce cost of goods

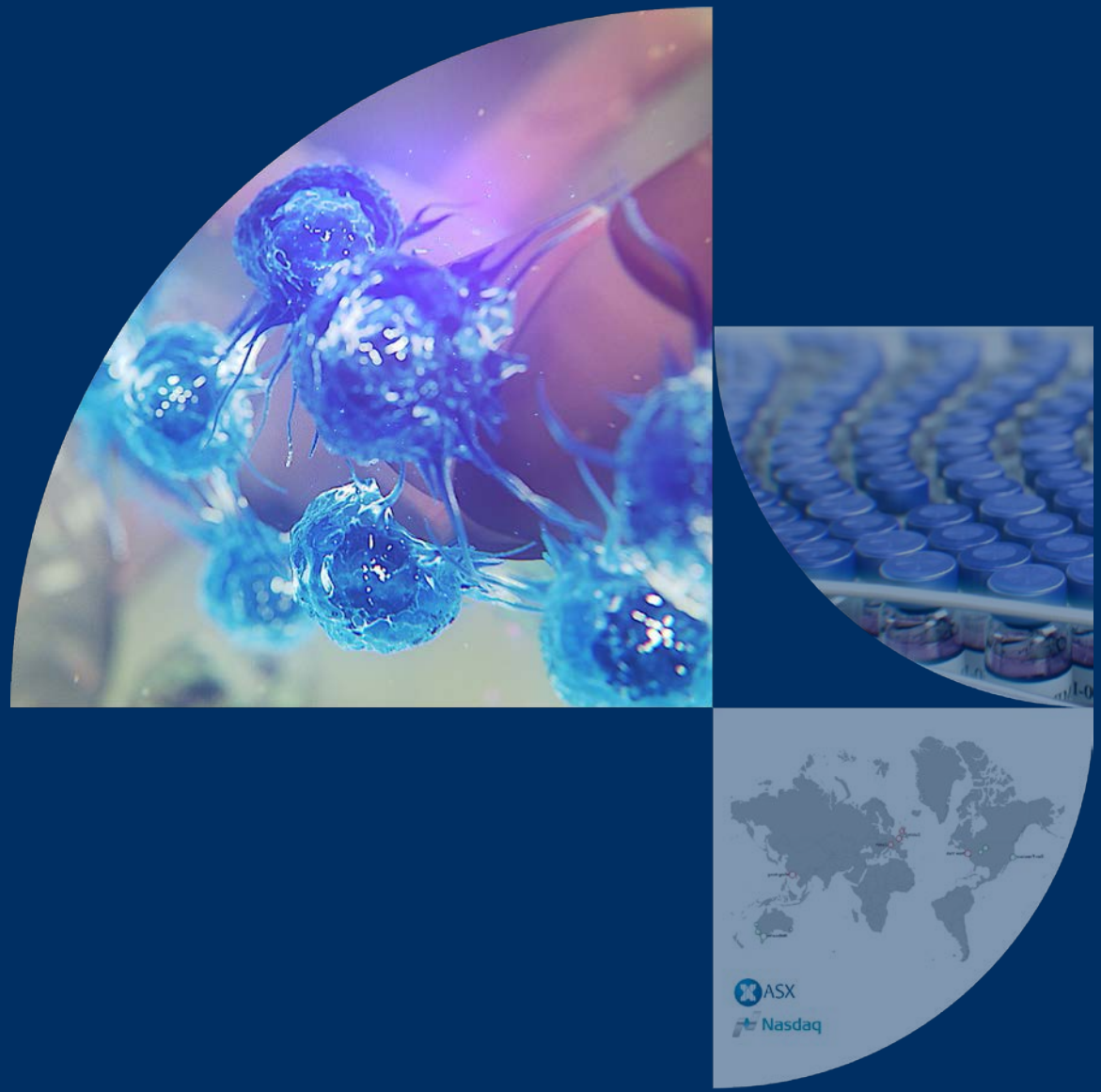
# Global IP Estate Provides Substantial Competitive Advantage

- Extensive patent portfolio with protection extending through 2040 in all major markets
- Over 1,000 patents and patent applications (68 patent families) across all major jurisdictions
- Covers composition of matter, manufacturing, and therapeutic applications of mesenchymal lineage cells
- Provides strong global protection in areas of our core commercial focus
- Grant rights to third parties who require access to our patent portfolio to commercialize their products, when outside our core commercial areas
- Mesoblast receives royalty income from its patent licensee TiGenix, S.A.U., a wholly owned subsidiary of Takeda, on its worldwide sales of its product Alofisel® for the treatment of complex perianal fistulas in adult patients with Crohn's disease, as well as milestone payments




Alofisel® is a registered trademark of TiGenix, S.A.U.

# Financials



# Financial Highlights

## First Nine Months FY2020 Compared to First Nine Months FY2019

- 113% increase in total revenue to US$31.5m from US$14.8m
- 81% growth in commercialization revenue from sales of TEMCELL to US$5.9m from US$3.3m
- 127% increase in milestone revenues from strategic partnerships to US$25.0m from US$11.0m
- 34% (US$23.7m) reduction in loss after tax
- 15% (US$7.5m) decrease in R&D spend

## Third Quarter FY2020 Compared to Third Quarter FY2019

- 10-fold increase in total revenue to US$12.2m from US$1.2m
- 99% growth in commercialization revenue from sales of TEMCELL to US$2.1m

Figures are rounded

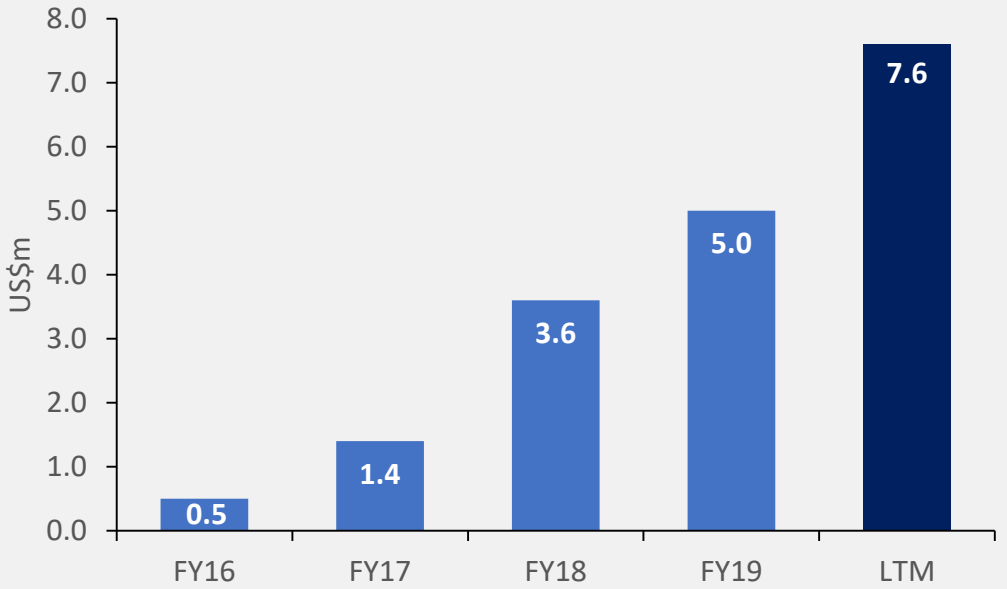
# Continued Growth in Revenues from Sales of TEMCELL in Japan for SR-aGVHD

## TEMCELL®

***ACUTE GRAFT VERSUS HOST DISEASE + OTHER INDICATIONS***

- JCR Pharmaceuticals has exclusive rights to Mesoblast's MSC technology for acute GVHD in Japan
- US$7.6 million royalties received in last 12 months
- Product adoption and reimbursement informs Mesoblast US commercial strategy for RYONCIL in acute GVHD
- US addressable market for acute GVHD in children and adults is ~ eight-fold larger than Japan due to greater patient numbers, incidence and pharmacoeconomics
- License expanded to cover:
  - Epidermolysis bullosa (EB), a highly debilitating and sometimes lethal skin disease; and
  - Hypoxic ischemic encephalopathy (HIE) in newborns, a disease with a high frequency of mortality

## ANNUAL REVENUE FROM TEMCELL ROYALTIES IN JAPAN




**Success of TEMCELL by Mesoblast Licensee JCR Informs Potential US Market for RYONCIL**

TEMCELL® Hs. Inj. is a registered trademark of JCR Pharmaceuticals Co Ltd.

# Substantial Increase in Revenues and Reduced Loss After Tax

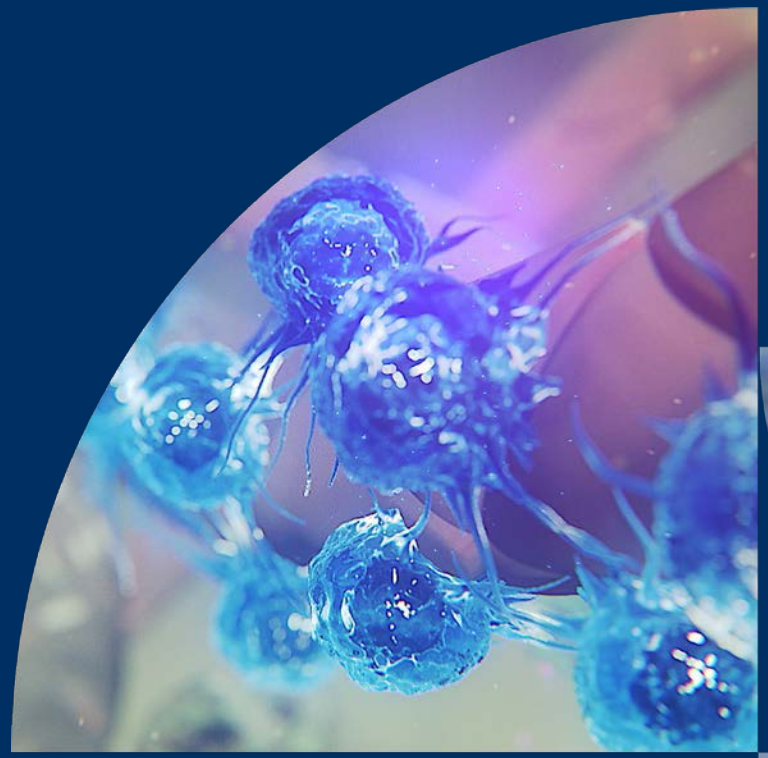
| Profit and Loss for the nine months ending (US$m) | March 31, 2020 | March 31, 2019 |
|---|---|---|
| Commercialization revenue | 5.9 | 3.3 |
| Milestone revenue | 25.0 | 11.0 |
| Interest revenue | 0.5 | 0.5 |
| **Total Revenue** | **31.5** | **14.8** |
| Research and development | (40.9) | (48.4) |
| Manufacturing | (15.5) | (12.9) |
| Management & administration | (18.0) | (16.0) |
| Contingent consideration | 1.3 | (3.4) |
| Other operating income & expenses | (0.0) | (1.1) |
| Finance costs | (9.9) | (7.9) |
| **Loss before tax** | **(51.5)** | **(74.9)** |
| Income tax benefit | 6.2 | 5.8 |
| **Loss after tax** | **(45.3)** | **(69.1)** |

Figures are rounded

# Strengthened Balance Sheet After Capital Raise

- Cash on hand at March 31, 2020 was US$60.1m
- Pro forma cash on hand is approximately US$150m, with the additional US$90m capital raised in May 2020
- Up to an additional US$67.5 million may be available through existing financing facilities and strategic partnerships over next 12 months
- Capital will be used for
  - Commercial launch of RYONCIL for acute GVHD
  - Scale-up of manufacturing for projected increase in capacity requirements for maturing pipeline, including GVHD label extensions and COVID-19 ARDS
  - Clinical programs supporting label extension strategies and regulatory approvals of Phase 3 assets

Figures are rounded




# RYONCIL (remestemcel-L): Acute Graft Versus Host Disease

# Acute Graft Versus Host Disease (aGVHD)

## Significant market opportunity for RYONCIL

**Burden of Illness**

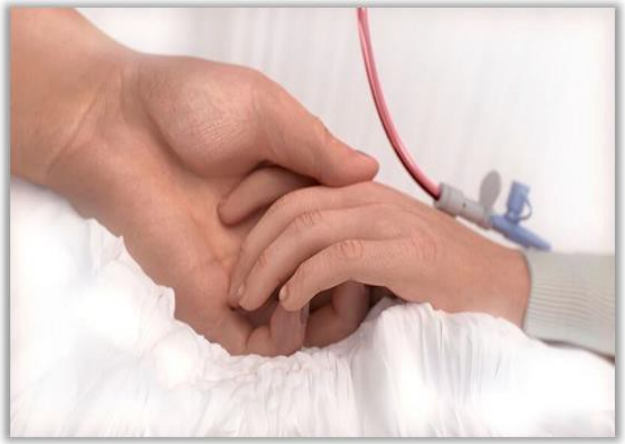
- aGVHD is a life-threatening complication that occurs in ~50% of patients receiving allogeneic bone marrow transplants (BMTs)[1]
- Steroid-refractory aGVHD is associated with **mortality rates as high as 90%[1,7] and significant extended hospital stay costs[2]**

**Minimal Treatment Options**

- There is only one approved treatment for SR-GVHD and **no approved treatment for children under 12 years old, outside Japan**
- In Japan, Mesoblast's licensee has received the only product approval for SR - aGVHD in both children and adults

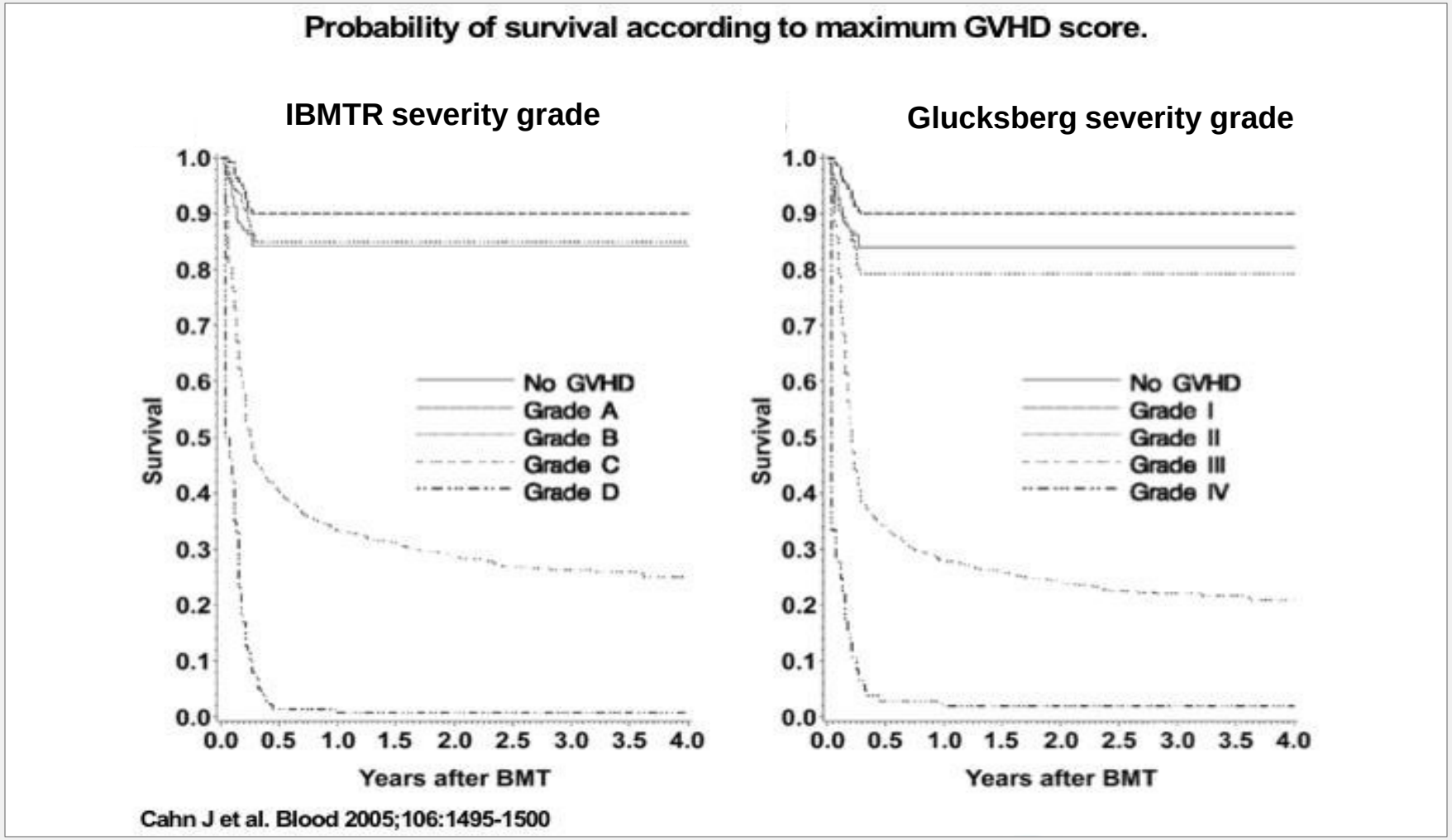
**Market Opportunity**

- >30,000 allogeneic BMTs performed globally (>20K US/EU) annually, ~20% pediatric[3,4]
- Our licensee JCR Pharmaceuticals Co., Ltd launched TEMCELL® HS Inj.[5] in Japan for SR-aGVHD in 2016; reimbursed up to ~$USD195k[6]
- **SR-aGVHD represents $USD > 700m US/EU market opportunity[4,8]**



**1.** Westin, J., Saliba, RM., Lima, M. (2011) Steroid-refractory acute GVHD: predictors and outcomes. Advances in Hematology. **2**. Anthem-HealthCore/Mesoblast claims analysis (2016). Data on file **3.** Niederwieser D, Baldomero H, Szer J. (2016) Hematopoietic stem cell transplantation activity worldwide in 2012 and a SWOT analysis of the Worldwide Network for Blood and Marrow Transplantation Group including the global survey. **4.** Source: CIBMTR Current Uses and Outcomes of Hematopoietic Cell Transplantation 2017 Summary. Passweg JR, Baldomero, H (2016) Hematopoietic stem cell transplantation in Europe 2014: more than 40,000 transplants annually. **5.** TEMCELL is the registered trademark of JCR Pharmaceuticals Co. Ltd. **6.** Based on a ¥JPY = $USD 0.009375 spot exchange rate on market close on November 11, 2016. Amounts are rounded. Source: Bloomberg. 7. Axt L, Naumann A, Toennies J (2019) Retrospective single center analysis of outcome, risk factors and therapy in steroid refractory graft-versus-host disease after allogeneic hematopoietic cell transplantion. Bone Marrow Transplantation. 8.Data on file

# Grade C/D GVHD has Significantly Worse Survival than Grade A/B



# RYONCIL: Phase 3 Trial compared to MAGIC Database

## Improved Day 28 Overall Response and Day 100 Survival relative to matched controls

- A comparative analysis performed between Mesoblast's open-label Phase 3 study and contemporaneous controls receiving institutional standard of care
- Phase 3 trial of RYONCIL (GVHD001) in 55 children, 89% of whom had Grade C/D disease
- A cohort of 30 pediatric patients with SR-aGVHD from the MAGIC consortium matched for inclusion criteria and disease severity (80% Grade C/D)

| Outcomes* | MSB-GVHD001 (n=54)[2] | MAGIC SR-aGVHD (n=30)[3] |
|---|---|---|
| Day 28 Overall Response | 70% | 43% |
| Day 100 Survival | 74% | 57% |

*rounded to nearest %

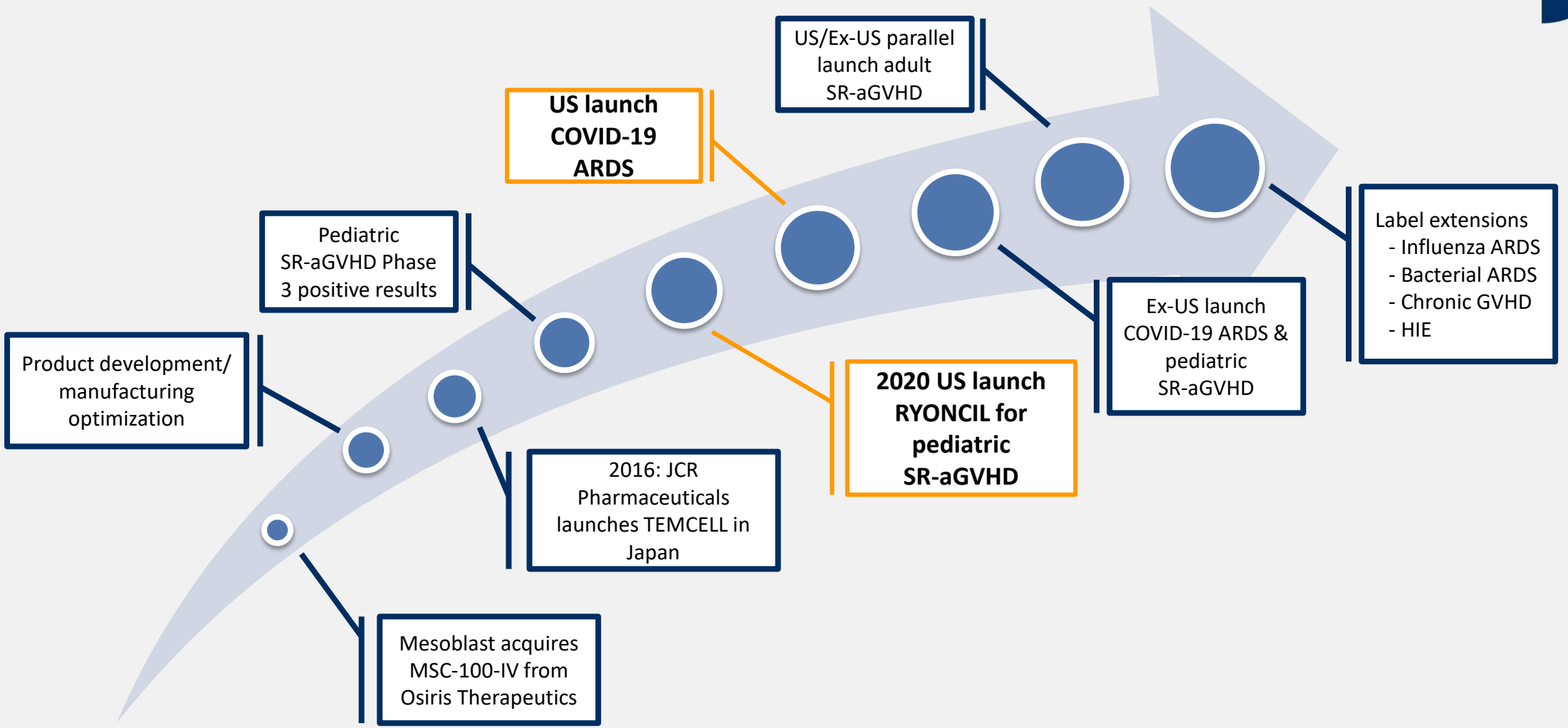
**RYONCIL has demonstrated efficacy and survival benefit in children with SR-aGVHD including those with the most severe grades of the disease**

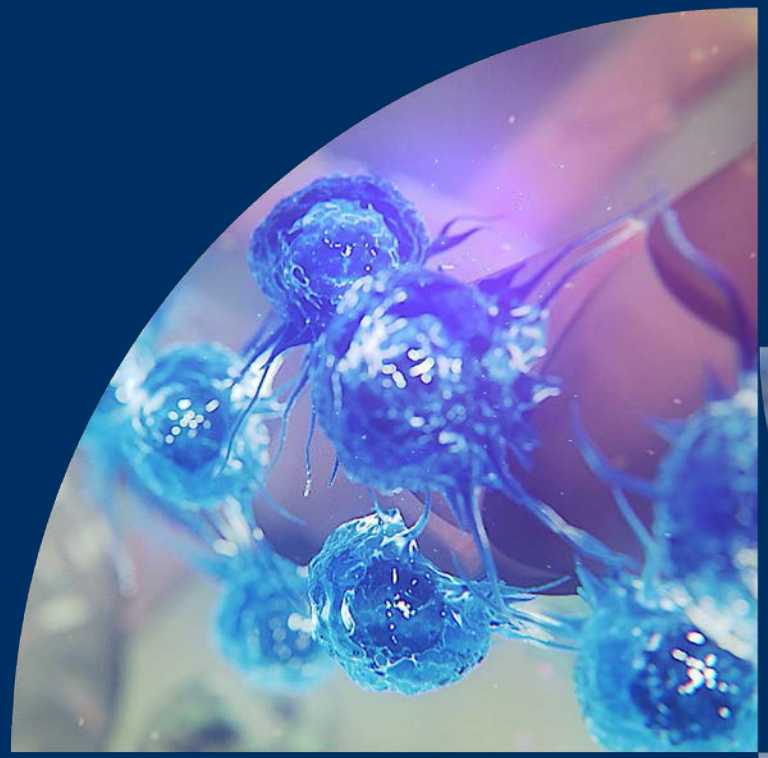
1. Mount Sinai Acute GVHD International Consortium (MAGIC) - a group of ten BMT centers throughout the US and Europe whose purpose is to conduct ground-breaking clinical trials in GVHD, including developing informative biorepositories that assist in developing treatments that can guide GVHD therapy.
2. GVHD001 had 55 randomized patients, however one patient dropped out before receiving any dose of remestemcel-L
3. Two subjects in the MAGIC cohort had follow-up <100 days; these subjects are excluded from the respective survival analyses.

# RYONCIL: Anticipated FDA Approval in 2020

- Results from three studies using RYONCIL in children and adults with SR-aGVHD support the FDA BLA filing
  - RYONCIL was used as salvage therapy in an expanded access program in 241 children with SR-aGVHD (80% Grade C/D) who failed institutional standard of care
  - RYONCIL was used as first-line therapy in Mesoblast's open-label Phase 3 trial in 55 children with SR-aGVHD, 89% of whom had Grade C/D disease
  - RYONCIL was used as first-line therapy in a randomized controlled Phase 3 trial of 260 adults and children with SR-aGVHD
- BLA filing for RYONCIL was accepted by the US FDA for priority review for the treatment of SR-aGVHD in children
- The FDA has set a Prescription Drug User Fee Act (PDUFA) action date of September 30, 2020
- If approved, RYONCIL launch in the US planned for Q4 CY2020

# Remestemcel-L: Lifecycle Extension Strategy



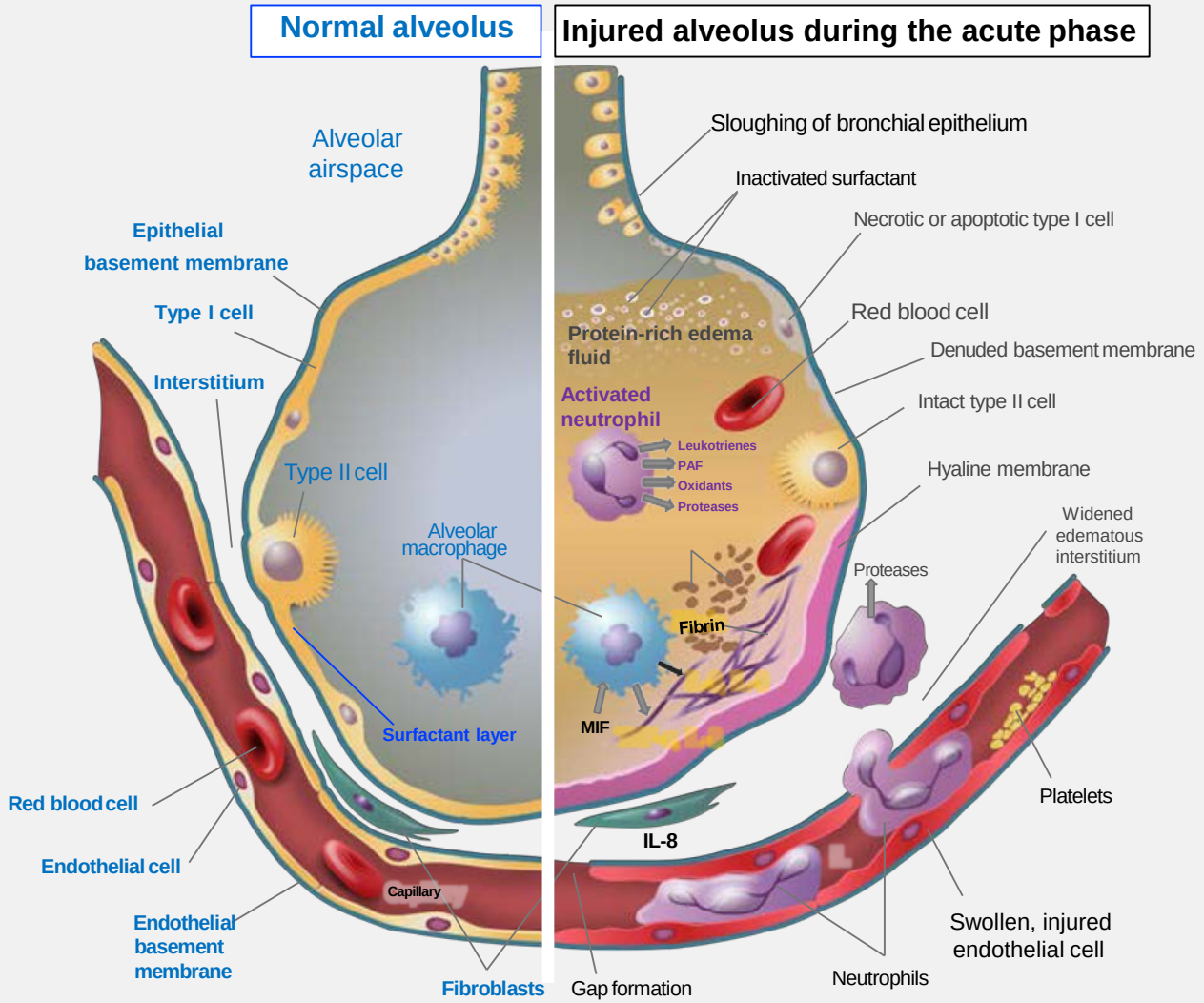



# Remestemcel-L:
# Acute Respiratory Distress Syndrome (ARDS) due to COVID-19

# Overview – Remestemcel-L for COVID-19 ARDS

- COVID-19 is a respiratory virus with a high mortality due to a severe inflammatory condition of the lungs called acute respiratory disease syndrome (ARDS)
- ARDS is caused by cytokine storm in lungs of patients infected with COVID-19 and is the primary cause of death
- The extensive safety data of remestemcel-L and its anti-inflammatory effects in aGVHD makes a compelling rationale for evaluating remestemcel-L in COVID-19 ARDS
- Intravenous delivery of remestemcel-L results in selective migration to the lungs making inflammatory lung disease an ideal target for this therapy
- Remestemcel-L has the potential to tame the cytokine storm in ARDS and may offer a life-saving treatment for those suffering from COVID-19

# ARDS due to COVID-19, Influenza & Bacterial Infection – Major Unmet Need




Source: Matthay MA, Zimmerman GA. Am J Respir Cell Mol Biol. 2005;33:319-27

## Acute respiratory distress syndrome (ARDS)

- A major area of unmet medical need
- Multiple triggers including viral/bacterial infections such as coronavirus or influenza
- Typically requires extended ICU hospitalization and intervention by ventilation
- ~40-80% mortality in viral induced ARDS (influenza & COVID-19, respectively)[1-4]

## Pathophysiology

- Activation of alveolar M1 macrophages results in cytokine storm
- Influx of neutrophils results in proteolytic destruction
- Aberrant secretion of fluid by alveolar cells
- Interstitial edema, cell death and influx of inflammatory cells

1. Matthay MA., et al. Acute Respiratory Distress Syndrome. Nature 2019 5:18. doi: 10.1038/s41572-019-0069-0; 2. Bellani G, Laffey JG, Pham T, et al. Epidemiology and patterns of care, and mortality for patients with acute respiratory distress syndrome in intensive care units in 50 countries. JAMA 2016;315:788-800; 3. Petrilli CM et al. Factors associated with hospitalization and critical illness among 4,103 patients with Covid-19 disease in New York City. MedRxiv 2020; 4. Gibson PG., et al. COVID-19 ARDS: clinical features and differences to "usual" pre-COVID ARDS. Med J Aust. 24 April 2020

# Rationale for Remestemcel-L Treatment of ARDS

- The COVID-19 virus stimulates a cytokine storm in the lung, increasing inflammatory cytokines and biomarkers such as TNFα, IL-6, IL-8, hepatocyte growth factor, and IL-2R leading to ARDS[1-3]
- When remestemcel-L arrives in the inflamed lung, its surface receptors are activated by major pro-inflammatory cytokines including TNFα and IL-6
- Engagement of these receptors results in secretion by remestemcel-L of multiple anti-inflammatory factors that switch off macrophages, B-cells and T-cells
- This results in reduction of the cytokine storm that causes ARDS and associated inflammatory biomarkers including TNFα, IL-8, hepatocyte growth factor, and IL-2R[4]
- The anti-inflammatory and additional reparative factors produced by remestemcel-L have the potential to reverse ARDS, protect alveolar epithelial cells, and improve lung function

1. Yuki K. et al. COVID-19 pathophysiology: A review. Clinical Immunology 215 (2020) 108427; 2. van de Veerdonk FL. et al. A systems approach to inflammation identifies therapeutic targets in SARS CoV-2 infection. medRxiv preprint doi: https://doi.org/10.1101/2020.05.23.20110916; 3. Gong J. et al. Correlation Analysis Between Disease Severity and Inflammation-related Parameters in Patients with COVID-19 Pneumonia. medRxiv preprint doi: https://doi.org/10.1101/2020.02.25.20025643; 4. Kutrzberg J. et al. A Phase 3, Single-Arm, Prospective Study of Remestemcel-L, ExVivo Culture-Expanded Adult Human Mesenchymal Stromal Cells for the Treatment of Pediatric Patients Who Failed to Respond to Steroid Treatment for Acute Graft-versus-Host Disease. Biol Blood Marrow Transplant Volume 26, Issue 5, May 2020, Pages 845-854

# Pilot Data From Emergency IND Provides Rationale for Randomized Controlled Phase 3 Trial of Remestemcel-L in COVID-19 ARDS

## Compassionate Use Data from Emergency IND

- 12 patients with moderate or severe ARDS received two infusions of remestemcel-L at Mt. Sinai Hospital in New York City
- Nine patients successfully came off ventilator support at a median of 10 days and were discharged from hospital
- This contrasts with only 9% of COVID-19 patients able to be extubated and a 12% survival rate in two major NY hospital networks during same time period[1,2]

## Confirmatory Phase 3 Trial

- Up to 300 patients randomized 1:1 to remestemcel-L or placebo
- Primary endpoint Day 30 mortality; Key secondary endpoint days alive off ventilator support
- First patients randomized and dosed in early May

1 Petrilli CM et al. Factors associated with hospitalization and critical illness among 4,103 patients with Covid-19 disease in New York City. MedRxiv 2020 doi: https://www.medrxiv.org/content/10.1101/2020.04.08.20057794v1.full.pdf
2. Richardson S et al. Presenting characteristics, comorbidities, and outcomes among 5700 patients hospitalized with COVID-19 in the New York City area. JAMA 2020. doi:10.1001/jama.2020.6775

# Phase 3 Trial of 300 Patients with ARDS due to COVID-19

**Objective:**

- Multi-center, randomized, controlled, blinded study to assess safety and efficacy of remestemcel-L versus standard of care (SOC) treatment in subjects with moderate/severe ARDS on ventilator due to COVID-19
- The trial will be conducted at up to 30 major teaching hospitals across North America

**Trial design:**

- 300 patients 1:1 randomized (150 SOC + remestemcel-L : 150 SOC + placebo)
- Dose is two infusions of remestemcel-L ($2x10^6$ cells/kg/dose) in the first week

**Primary endpoint:** all cause mortality up to 30 days post randomization

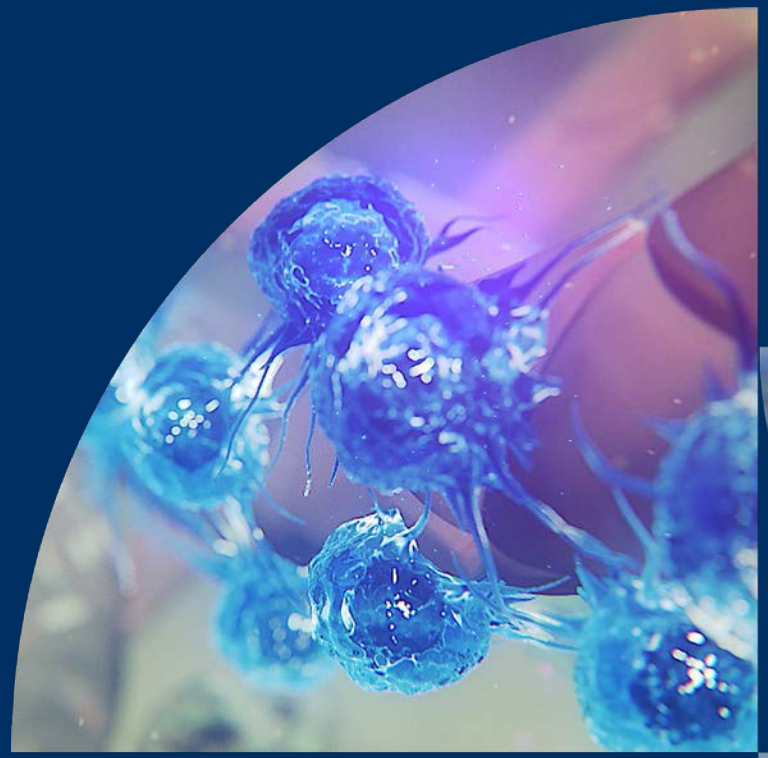
**Key secondary endpoint:** days alive off ventilator within 60 days

**Additional information:**

- Recruitment is expected to complete within three to four months, with interim analyses planned which could result in stopping the trial early for efficacy or futility

# Key Milestones for Remestemcel-L in COVID-19 ARDS

- Recruitment is expected to take three to four months
- Interim analyses planned which could result in stopping the trial early for efficacy or futility. First interim analysis when 30% of patients reach the primary endpoint
- Seek expedited regulatory approval subject to positive data read-out
- Manufacturing scale-up to meet projected increase in capacity requirements for maturing pipeline, including GVHD label extensions and COVID-19 ARDS
  - Increase manufacturing footprint for capacity expansion
  - Implement proprietary xeno-free technologies to increase yields and output
  - Plan for long-term move to 3D bioreactors to reduce labor and improve manufacturing efficiencies
- Establish manufacturing and commercialization partnerships




## Update on Phase 3 Product Candidates

- Heart Failure
- Chronic Low Back Pain

# Partnerships and License Agreements

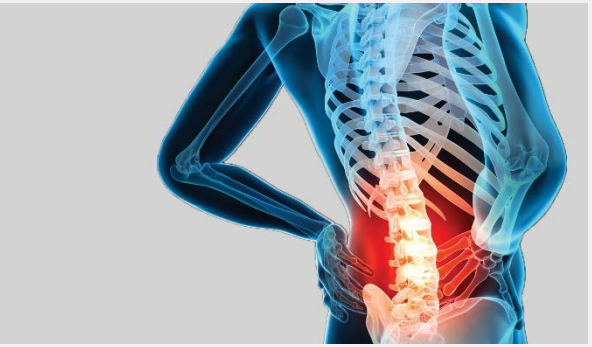
## Phase 3 Product Candidates

### MPC-06-ID

- Strategic partnership to develop and commercialize MPC-06-ID in Europe & Latin America
- Mesoblast will receive up to US$150 million in upfront and milestone payments prior to product launch
- Milestone payments could exceed US$1 billion depending on patient adoption
- Mesoblast will also receive tiered double digit royalties on product sales

***CHRONIC LOW BACK PAIN - DEGENERATIVE DISC***

**PREVALENCE EUROPE**
**~7.0 MILLION**

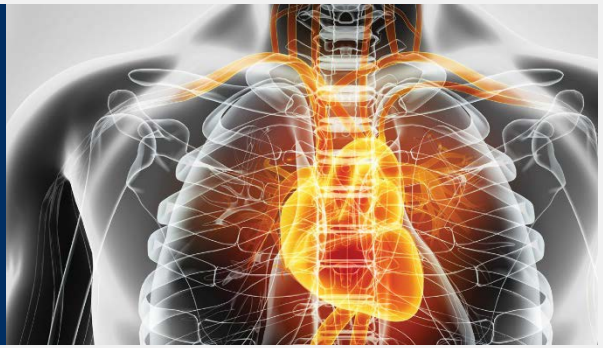


### REVASCOR™

- Exclusive cardiovascular rights in China
- Mesoblast received US$40 million in an upfront payment and equity placement
- Eligible for additional milestones and royalties

***CARDIOVASCULAR – CHRONIC HEART FAILURE***

**PREVALENCE CHINA**
**~4.5 MILLION**



# REVASCOR for Advanced and End-Stage Heart Failure

- In December 2019, the Phase 3 trial in advanced heart failure surpassed the number of primary endpoint events required for trial completion
  - Final study visits for all surviving patients have been completed
  - Ongoing quality review of all data is being completed at the study sites
  - Data readout planned for mid-2020
  - Results may support regulatory approval in the US
- Results from a sub-study of 70 patients with end-stage ischemic heart failure and a Left Ventricular Assist Device (LVAD), of 159 randomized patients who received either Mesoblast's product candidate Revascor® or saline, were presented at the American College of Cardiology (ACC) Virtual Scientific Sessions
  - Conclusions from the study included MPCs had a beneficial effect on LVAD weaning, major mucosal bleeding, serious adverse events, and readmissions in ischemic heart failure patients
  - End-stage ischemic heart failure patients with LVADs are older and have co-morbidities such as diabetes, thereby closely resembling the majority of patients in Mesoblast's 566-patient Phase 3 trial of Revascor for advanced chronic heart failure

# MPC-06-ID for Chronic Low Back Pain

- Phase 3 trial of MPC-06-ID for chronic low back pain in 404 patients:
  - Final study visits for all patients have been completed
  - Ongoing quality review of all data is being completed at the study sites
  - Data readout planned for mid-2020

- Continued operational progress in strategic partnership for chronic lower back pain with Grünenthal in Europe to complete clinical protocol design, obtain regulatory input, and receive clearance from European regulatory authorities to begin European Phase 3 trial

- Results from the Phase 3 trials will be considered pivotal to support regulatory approval in the US, as well as in Europe

# Major Operational Milestones for the Next 12 Months

**Remestemcel-L for SR-aGVHD & Other Rare Diseases**

- RYONCIL Priority Review underway with PDUFA date set for September 30, 2020
- If approved, US launch of RYONCIL planned for 2020
- Expand investigator-initiated clinical trials for chronic GVHD and other indications

**Remestemcel-L for Acute Respiratory Distress Syndrome (ARDS) in COVID-19**

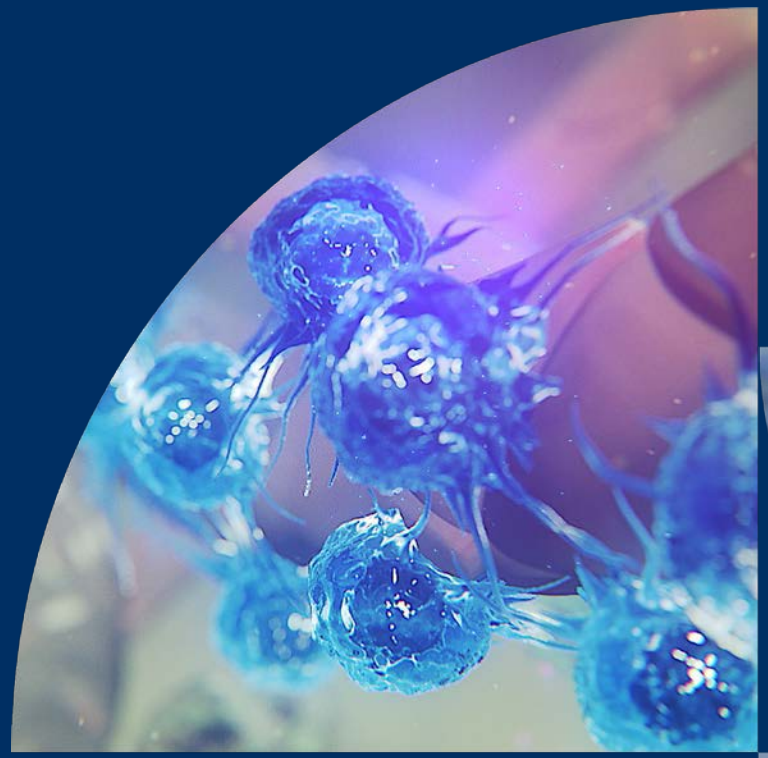
- Ongoing recruitment for Phase 3 multicenter, randomized controlled trial in North America
- Trial completion expected in approximately 3-4 months
- Establish strategic partnerships for manufacturing and commercialization

**REVASCOR for Advanced and End-Stage Heart Failure**

- Data readout for advanced chronic heart failure Phase 3 trial in mid-2020
- Initiate confirmatory trial in end-stage heart failure

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

- Data readout for Phase 3 trial in mid-2020
- Obtain clearance from European regulatory authorities to begin European Phase 3 trial



mesoblast


# Thank You