



# **2018 Annual General Meeting**

## **Dr Silviu Itescu, Chief Executive**

November 30, 2018

Nasdaq: MESO ASX: MSB

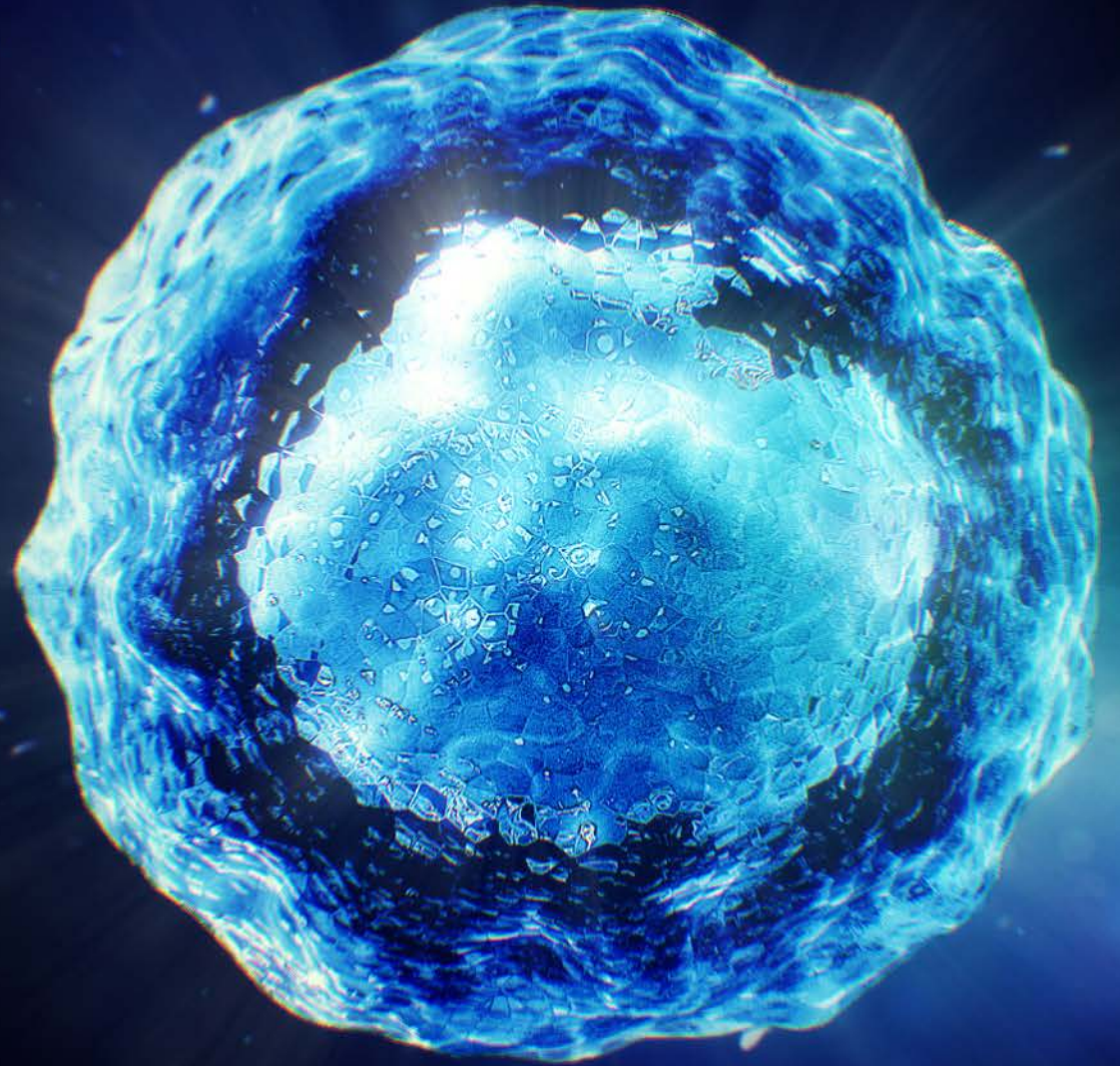


## CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

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# Our Mission

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



# Premier Global Cellular Medicines Company










Disruptive Technology Platform <sup>1</sup>	Commercialization	Late Stage Pipeline
<ul style="list-style-type: none"><li>▪ Disruptive technology which targets the most severe disease states refractory to conventional therapies</li><li>▪ Well characterized multimodal mechanisms of action</li><li>▪ Underpinned by extensive, global IP estate</li></ul>	<ul style="list-style-type: none"><li>▪ First approved products commercialized by licensees in Japan<sup>2</sup> and Europe<sup>3</sup></li><li>▪ Increasing revenues and milestone payments</li><li>▪ Industrial-scale manufacturing to meet commercial demand</li><li>▪ Building focused U.S. sales force for upcoming GVHD product launch</li></ul>	<ul style="list-style-type: none"><li>▪ 2 blockbuster product candidates in heart failure and back pain Phase 3 trials</li><li>▪ Upcoming FDA interactions under RMAT regarding Phase 2 LVAD trial results</li><li>▪ China cardiovascular partnership in place</li><li>▪ Global partnership discussions to leverage commercial capabilities</li></ul>

1. Mesenchymal precursor cells (MPCs) and their culture-expanded progeny mesenchymal stem cells (MSCs).

2. 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.

3. Licensee Takeda received first central marketing authorization approval from the European Commission for an allogeneic stem cell therapy and markets this product under its trademark, Alofisel®.

# Multiple Commercial Opportunities

PLATFORM		PRODUCT	THERAPEUTIC AREA				APPROVAL	COMMERCIAL RIGHTS	MARKETED	
MSC (Bone Marrow)		TEMCELL® HS Inj <sup>1</sup>	Acute Graft Versus Host Disease	1st allogeneic regen med approved in Japan			✓	 Japan		
MSC (Adipose)		Alofisel® <sup>2</sup>	Perianal Fistula	1st allogeneic regen med approved in Europe			✓	 Global		
PLATFORM		PRODUCT CANDIDATE	THERAPEUTIC AREA	PRE-CLINICAL	PHASE 2	PHASE 3	COMMERCIAL RIGHTS			IN DEVELOPMENT
TIER 1	MSC	Remestemcel-L	Acute Graft Versus Host Disease				 the regenerative medicine company			
	MPC	Revascor	Advanced HF (Class II/III) End-Stage HF (Class III/IV) <sup>3</sup>				 the regenerative medicine company  China <sup>4</sup>			
	MPC	MPC-06-ID	Chronic Low Back Pain				 the regenerative medicine company			
	MPC	MPC-300-IV	Rheumatoid Arthritis Diabetic Nephropathy				 the regenerative medicine company			
TIER 2	Includes remestemcel-L (Crohn's disease – biologic refractory), MPC-25-IC (Acute Cardiac Ischemia), MPC-25-Osteo (Spinal Fusion) and MPC-75-IA (Knee Osteoarthritis)									

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

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

# Partnerships and License Agreements



- JCR has rights to use our MSC technology to treat acute GVHD in Japan
- Its product, TEMCELL<sup>®</sup> HS Inj., was the first fully approved allogeneic cellular medicine in Japan
- Royalties and milestones received in last twelve months exceed US\$5 million
- License expanded in Oct 2018 to cover use in treatment of epidermolysis bullosa – a highly debilitating and sometimes lethal skin disease



- Patent license agreement entered in Dec 2017 with Takeda (formerly TiGenix NV) providing exclusive access to certain IP for local treatment of perianal fistulae
- Mesoblast is eligible to receive €20 million in milestone payments plus royalties upon commercial sales of Alofisel<sup>®</sup> worldwide



- Exclusive cardiovascular rights in China
- Mesoblast received US\$40 million on closing, eligible to receive additional milestones and royalties
- Tasly expects to meet with China's regulatory authority in early 2019 to discuss a pathway for approval of Mesoblast heart failure cell therapy in China



# Global IP Estate Provides Substantial Competitive Advantage

- ~800 Patents and patent applications (69 Patent families) across all major jurisdictions
- Covers composition of matter, manufacturing, and therapeutic applications of mesenchymal lineage cells
- Enables licensing to third parties for different indications, when in alignment with our corporate strategy, e.g. TiGenix (subsequently acquired by Takeda)
- Provides strong global protection against competitors seeking to develop products in areas of core commercial focus



# Commercial-Scale Manufacturing Capability

- Immune privileged nature of mesenchymal lineage cells enables allogeneic “off the shelf” product candidates
- Culture expansion scalable to produce anticipated commercial quantities
- Management know-how in regulatory activities necessary for product approval and commercial launch



*Lonza contract manufacturing facility in Singapore*

**We believe remestemcel-L will be the first commercially produced allogeneic mesenchymal lineage cell product registered for sale in the U.S.**



# Commercial Organizational Transition



## **Board of Directors – Structured Succession Plan to Bring Complementary Skills:**

- Proven FDA product approval capabilities
- Commercial launch expertise
- Reimbursement and health system expertise
- Extensive global transactional record

## **Management – Expand Know-How to Support Commercial Launch Plans:**

- Commercial leadership with proven track record to roll out launch team
- Operational leadership to drive product life cycle management, commercial manufacturing and regulatory interactions

# Remestemcel-L: Graft Versus Host Disease

## Pathway to Market

- TEMCELL® HS Inj. sales experience in Japan informs commercial strategy for the U.S.
- Phase 3 successfully completed
- Fast Track designation provides eligibility for FDA priority review
- Commercialization strategy in place for product launch
- Building out efficient, targeted sales force
- \$700m US/EU addressable market, no competing approved products

**FDA Biologic License Application filing planned Q1 2019**

# Revascor: Targeting Patients with Advanced Heart Failure Refractory to Standard of Care

## Common Treatment Pathway in Progressive Heart Failure<sup>1</sup>

*Early*

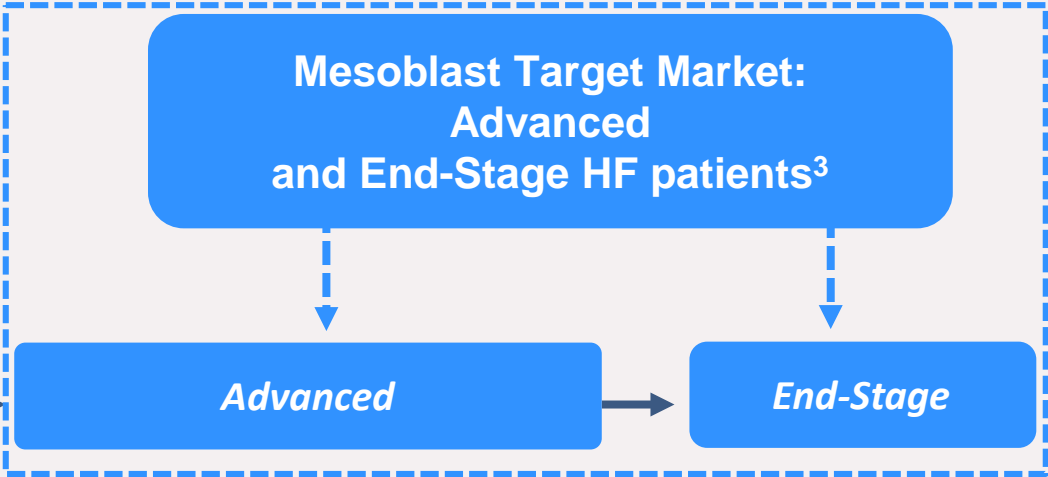
- ACEI or ARB
- Statins
- Beta blockers
- Re-vascularization or valvular surgery

*Pharmacological Add-on*

- Diuretics for fluid retention
- Aldosterone antagonists
- Hydralazine / isosorbide dinitrate
- Digitalis

*New Oral Therapies for Class II-IV<sup>2</sup>*

- If ACEI / ARB tolerated, sacubitril/valsartan



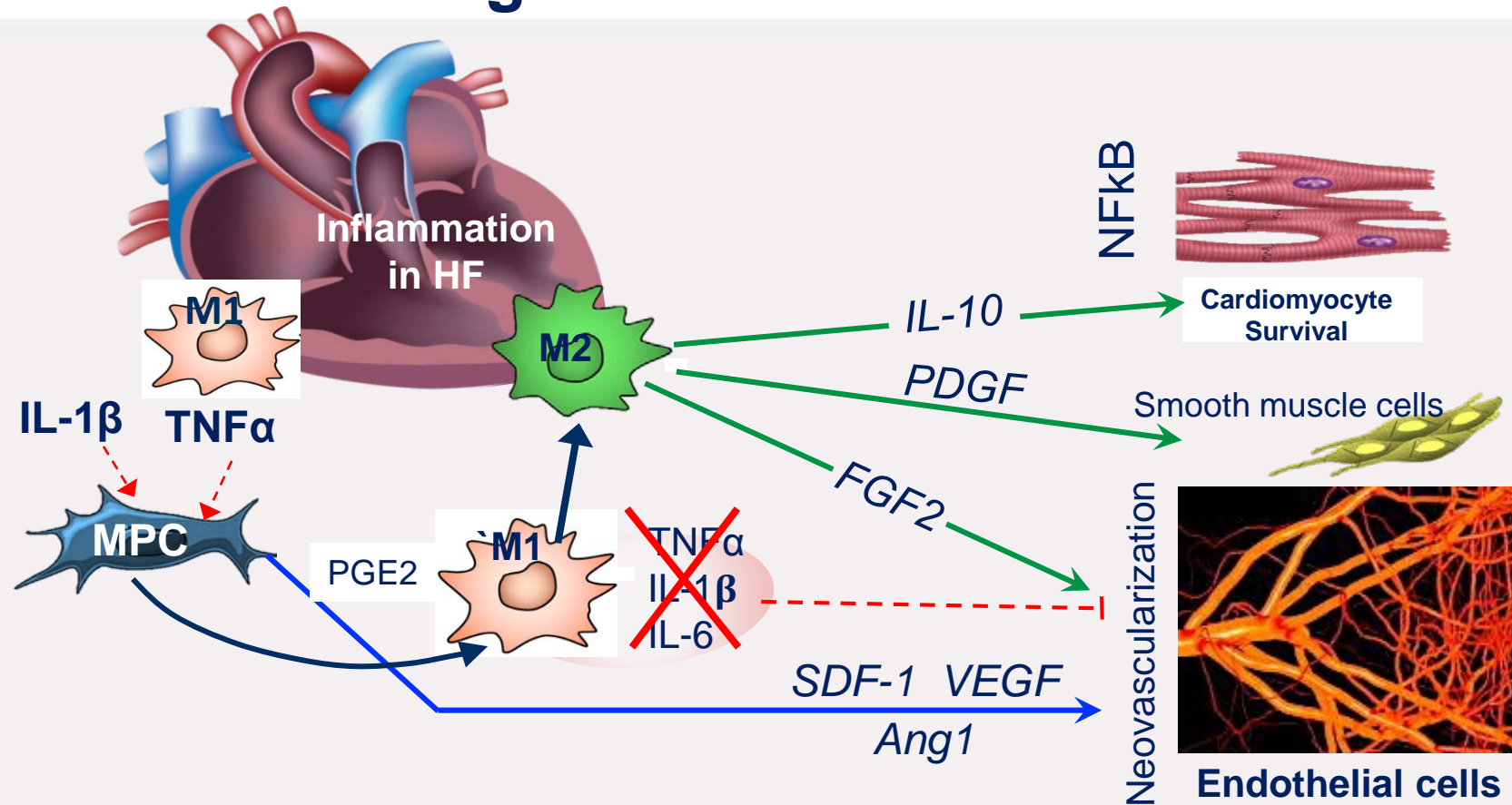
Class I

Heart Failure Disease Progression

Class IV

1. Source: Simon-Kucher & Partners 2017. Primary research 2017; Payers n=35, KOLs n=15, Cath lab managers n=4.  
2. Corlanor® (ivabradine) approved by FDA (April 2015). ENTRESTO® (sacubitril/valsartan) approved by FDA (July 2015).  
3. GlobalData-PharmaPoint Heart Failure (2016); McMurray et al., 2012; Yancy et al., 2013, 2016 ACC/AHA/HFSA Focused Update on New Pharmacological Therapy for Heart Failure: An Update of the 2013 ACCF/AHA Guideline for the Management of Heart Failure.

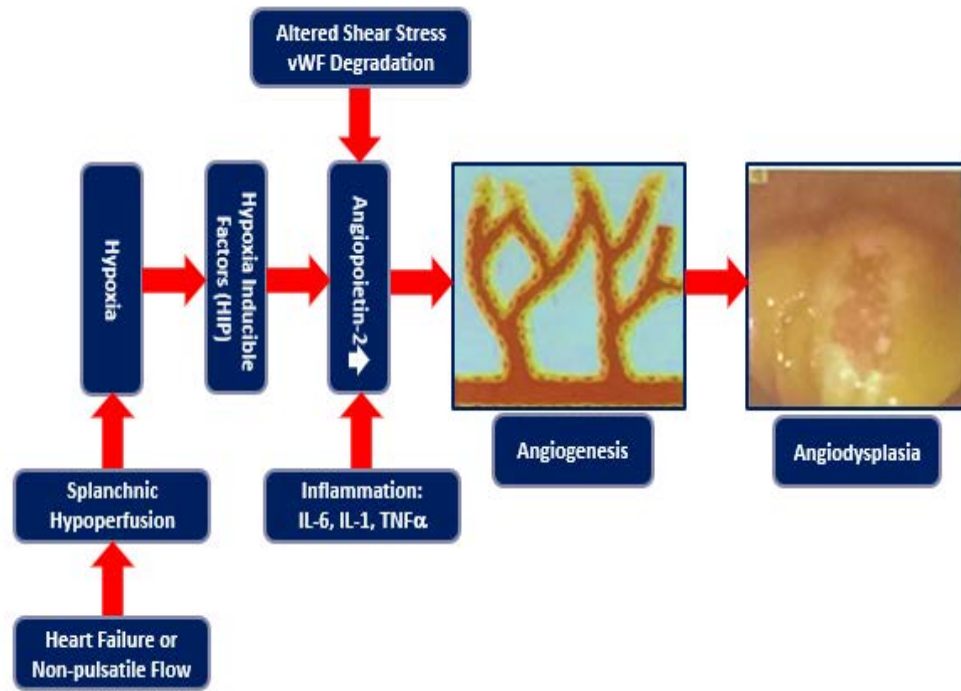
# Blood Vessel (Endothelial) Dysfunction in the Heart and Major Organs is at the Core of Progressive Heart Failure



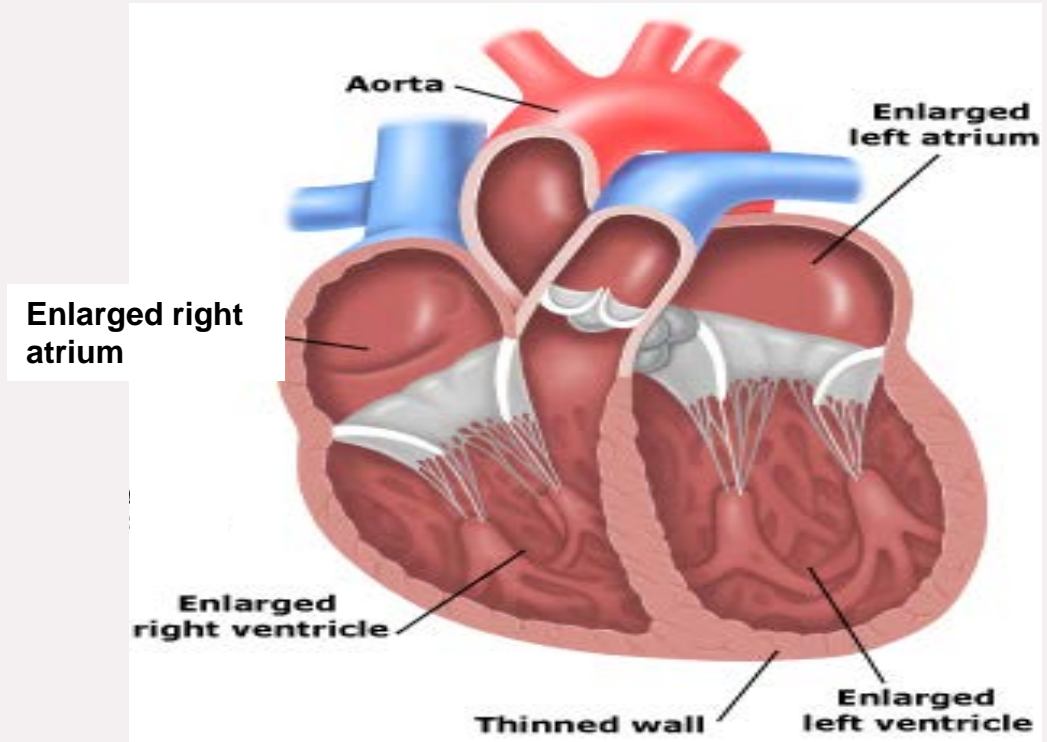
**Mesoblast MPCs have been shown to reverse inflammation-related endothelial dysfunction<sup>1</sup>**

# GI Bleeding in LVAD Patients due to Inflammation-Related Endothelial Dysfunction

## GI Blood Vessels are Abnormal in LVAD Patients



## Right Ventricular Failure and Increased Pressure in GI Blood Vessels



**Mesoblast MPCs significantly reduced GI bleeding in two randomized controlled clinical trials in LVAD patients**



# Revascor: End-stage LVAD Heart Failure Program

## Pathway to Market



- In end-stage heart failure patients with LVADs, inflammation-related endothelial dysfunction results in severe GI bleeding and recurrent hospitalizations
- Treatment of end-stage heart failure patients with our MPCs in two NIH funded studies showed a reduction in GI bleeding and related hospitalizations
- FDA guidance has indicated that GI bleeding associated with LVADs is a clinically meaningful outcome
- Mesoblast has received RMAT designation for use of Revascor as an adjunct to LVAD implantation, enabling eligibility for FDA priority review and accelerated approval
- > \$500m (US only) addressable market

**Plan to meet with FDA in H1 2019 to discuss potential approval pathway**

# Blockbuster Product Opportunities

## Revascor: Advanced Heart Failure

- New therapies needed to reduce hospitalizations and mortality
- Phase 3 trial in advanced heart failure has enrolled >90% of patients
- Majority of patients in this Phase 3 trial have ischemic heart failure
  - Revascor significantly improved LVAD wean tolerance and reduced hospitalizations from GI bleeding in ischemic heart failure patients in NIH-funded Phase 2 trial

## MPC-06-ID: Back Pain

- Limited treatment options for patients who fail conservative therapy include opioids and surgery
  - 50% of opioid prescriptions are for chronic low back pain
  - Opioid crisis is associated with a high rate of overdose and accidental death
  - Urgent need for novel therapies to avoid opioid use
- Phase 3 study completed enrollment ~400 patients in March 2018

# Global Partnerships to Commercialize Blockbuster Assets



- Advanced discussions with multiple potential partners for global commercialization of cell therapies for:
  - advanced heart failure
  - chronic low back pain due to disc degeneration
- Partners will bring:
  - access to high growth markets
  - clinical, regulatory and manufacturing expertise
  - established extensive commercial footprint

**Global pharma partnering discussions fueled by positive late-stage trial results, regulatory interactions and regional licenses**

# Significant Increase in Revenue

Revenue for the quarter ending September 30, 2018 (US\$m)

For the quarter ending	September 30, 2018	September 30, 2017	\$ Change	% Change
Milestone revenue	10.5	0.5	10.0	NM
Commercialization revenue	1.0	0.6	0.4	66%
Interest revenue	0.2	0.1	0.1	93%
<b>Total revenue</b>	<b>11.6</b>	<b>1.2</b>	<b>10.5</b>	<b>NM</b>

## First quarter FY2019 revenue increased by US\$10.5 million vs 2018 revenue due to:

- Commercialization revenue from royalty income on sales of TEMCELL®<sup>1</sup> HS. Inj. increased 66% for the quarter and 116%<sup>2</sup> for the 12 months ended September 30, 2018 compared to the 12 months ended September 30, 2017
- US\$10 million of milestone revenue in relation to establishing a strategic cardiovascular partnership with Tasly in China

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

2. Growth reported in constant currency which eliminates the effects of fluctuations in foreign exchange rates between different reporting periods.

# Cash Position Strengthened through Strategic Transactions

Balance sheet cash (US\$m)

	September 30, 2018	June 30, 2018	\$Change
Reported cash on hand	55.1	37.8	17.3
NovaQuest financing agreement	-	39.0	(39.0)
Tasly strategic partnership	40.0	40.0	-
<b>Pro forma cash on hand</b>	<b>95.1</b>	<b>116.8</b>	<b>(21.7)</b>

- Pro forma cash on hand at September 30 includes US\$40 million received in October 2018 on closing of the strategic cardiovascular partnership with Tasly previously announced in July 2018
- An additional US\$50 million may be available under existing arrangements with Hercules Capital and NovaQuest, subject to achievement of certain milestones



# Corporate Milestones



## **Remestemcel-L for Acute Graft Versus Host Disease**

- Successfully met all efficacy and safety endpoints through six months ✓
- FDA meetings and BLA filing (Q4 CY18 – Q1 CY19)

## **Revascor for Advanced and End-Stage Heart Failure**

- Meet with FDA to discuss the recent LVAD phase 2b study results for the clinically meaningful GI bleeding data (1H CY19)
- Phase 3 events-driven trial in advanced heart failure enrollment completion (Q4 CY18 – Q1 CY19)

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

- Phase 3 trial completed enrollment (Q1 CY18) ✓

**Completed non-dilutive transactions for commercialization of MSC-100-IV (remestemcel-L) ✓**

**Establish regional strategic and commercial licensing arrangements (China, Japan, Europe) ✓**

**Establish global commercial partnerships; in advanced discussions on blockbuster products**



Questions?



# **Commercial Launch Plans**

## **Eric Strati Pharm.D., MBA**

November 30, 2018

Nasdaq: MESO ASX: MSB

# Mesoblast is Committed to Patient Access and Maximizing Value for all Key Stakeholders

## Mesoblast Culture

- Highly driven and passionate organization focused on improving the lives of those suffering from life threatening diseases

## Experienced Commercial Team

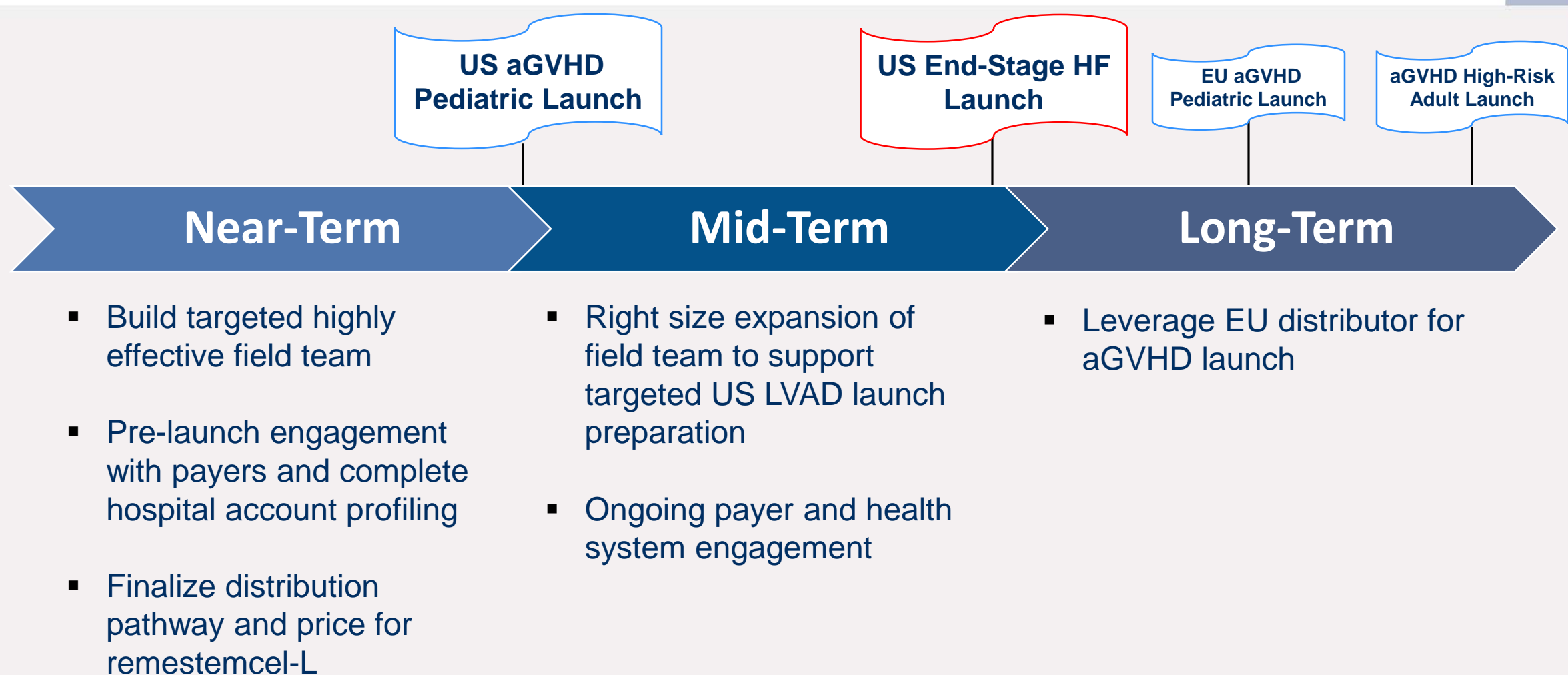
- High commercial acumen underpinned by strong scientific foundation and knowledge of reimbursement dynamics within health systems / transplant centers

## Targeted Commercial Objectives

- Optimize patient access while realizing the full value of Mesoblast products
- Provide effective product education and distribution support

# Mesoblast Planned Commercial Launch Activities for Lead Products

Targeted for > \$1bn Market Combined<sup>1,2,3</sup>



<sup>1</sup> Sources for GVHD Market Assessment: CIBMTR Current Uses and Outcomes of Hematopoietic Cell Transplantation 2017 Summary., Passweg JR, Baldomero, H (2016) Hematopoietic Stem Cell Transplantation in Europe 2014., Data on file, <sup>2</sup> Sources for LVAD Market Assessment: Agency for Healthcare Research and Quality – Healthcare Cost and Utilization Project- <https://www.ahrq.gov/data/hcup/index.html> – ICD-9 37.6., Data on file, <sup>3</sup> Subject to FDA discussions under existing RMAT designation



# Remestemcel-L: US Launch Strategy to Maximize Patient Access with Limited Investment



## Pre-Launch Activities

- Engage with key payers pre-launch to solidify market access and pricing strategy; support with appropriate health care economic information
- Pricing and Reimbursement driven by cost savings from reduction in length of hospital stay and improved survival<sup>1,2,3</sup>

## Launch Activities

- Target top 15 centers which represent ~50% of patient volume<sup>4</sup>
- Support access through patient centric program

<sup>1</sup>, 2016 claims analysis with Anthem/HealthCore – Data on File, <sup>2</sup> Data on file, <sup>3</sup> Mesoblast remestemcel-L study 001 trial results

<sup>4</sup> GVHD Market Assessment: CIBMTR Current Uses and Outcomes of Hematopoietic Cell Transplantation 2017 Summary

# Results from Providers/Payers Qualitative US Market Research<sup>1</sup>



(n=20)

0

Reaction to  
Tested Target Profile<sup>2</sup>

Median  
Response

6

7

Max Rating Product  
Attributes

## Most Significant Value Drivers for Remestemcel-L

- Day 100 Survival rate
- Day 28 overall response rate
- No increase in infections
- Largest clinical data set (n ~300)
- Ability to administer the drug outpatient

**“Remestemcel-L is Expected to  
Become Standard of Care”**

**- Multiple Respondents<sup>1</sup>**

<sup>1</sup> ZS Associates June 2018 Qualitative Market Research: MCO Medical Directors n=5, Transplant Center Directors n= 5, Hospital Pharmacy Directors n=5, AMC-based Hem/Oncs / KOLs n=3

<sup>2</sup> Tested Target Profile reflective of Mesoblast phase III study 001 results

# Revascor: Adjunct Therapy to LVAD Indication Commercial Strategy will Leverage Commercial Infrastructure



## Pre-Launch Activities

- Engage with key payers pre-launch to develop and solidify market access and pricing strategy
- Pricing and Reimbursement driven by cost savings from reduced GI-bleed hospitalizations and associated co-morbidities
- Apply for New Technology Add-on Payment (NTAP) program under which Centers for Medicare and Medicaid Services provides additional payments for qualified new technologies provided in the inpatient setting.

## Launch Activities

- Primarily target top 40 centers at launch representing ~75% of patient volume<sup>1</sup>
  - ~20% overlap with Tier 1 target transplant centers for aGVHD<sup>2</sup>

<sup>1</sup> Medicare provider inpatient charge data-FY2016 - <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Medicare-Provider-Charge-Data/Inpatient2016.html>

<sup>2</sup> <https://www.cms.gov/Medicare/Medicare-Fee-for-Service-Payment/AcuteInpatientPPS/newtech.html>



Questions?