

## **Safe Harbor Statement**

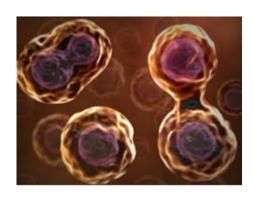
This presentation contains forward-looking statements that involve risks and uncertainties. These statements may concern, among other things, our business strategy and market opportunities, the development of pharmaceutical products, and future financial and operating results.

Additional information that may affect our business and financial prospects, as well as factors that would cause our actual performance to vary from our expectations, may be found in our filings with the Securities and Exchange Commission.



# Mesenchymal Precursor Cell (MPC) License From Mesoblast Limited

MPCs produce cytokines and/or growth factors that induce endogenous tissue repair or cell proliferation



#### **Promising early data with:**

- Congestive Heart Failure phase 2
- Myocardial Infarction pre-clinical
- Bone marrow transplant (cord blood expansion) phase 2



# Single Intra-Myocardial Injection Of Allogeneic MPC For Long-Term Treatment of Congestive Heart Failure

## Phase 2a Congestive Heart Failure (CHF) Clinical Trial

- 60 patient multi-center, randomized, controlled trial
- Class II-IV CHF with EF < 40%</li>
- Randomized 3:1 controls to MPC at 25 M, 75 M, or 150 M cell doses
- Cells injected by JNJ NOGA Myostar™ catheter
- Primary endpoint of safety already met:
   no adverse events associated with MPC at any dose
- Secondary endpoints evaluate effects of MPC on
- (a) cardiac/heart failure hospitalization events over time
- (b) cardiac-related mortality over time
- (c) cardiac functional parameters after patients complete 12 months



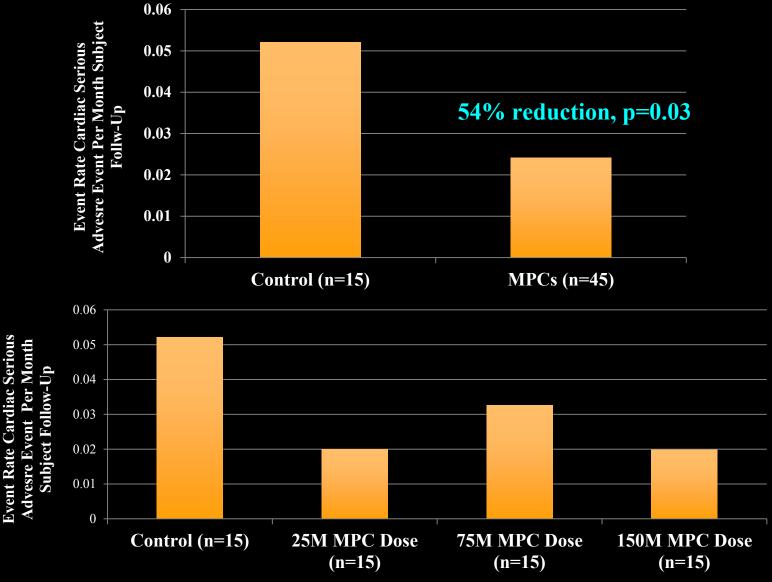
# Over 1.5 Years Study Follow-Up, MPC Treated Patients Had Fewer Cardiac-Related Events, Hospitalizations, And Deaths Than Controls

Event	MPC treatment (N=45) No. patients with event (%)	Controls (N=15) No. patients with event (%)	p value
Any Serious Adverse Cardiac Event (SAE)	20 (44.4%)	14 (93.3%)	0.001
Repeat SAEs	5 (11.1%)	5 (33.3%)	0.102
Any Hospitalization For Heart Failure	5 (11.1%)	3 (20.0%)	0.4
All Cause Deaths	2 (4.4%)	2 (13.3%)	0.26
Cardiac Deaths	0 (0.0%)	2 (13.3%)	0.059
Any Major Adverse Cardiac Event (MACE*)	3 (6.7%)	6 (40%)	0.005
MACE or Any Hospitalization for Heart Failure	6 (13.3%)	6 (40%)	0.056

Interim data analysis December 2010, after all patients have reached 6 months follow-up



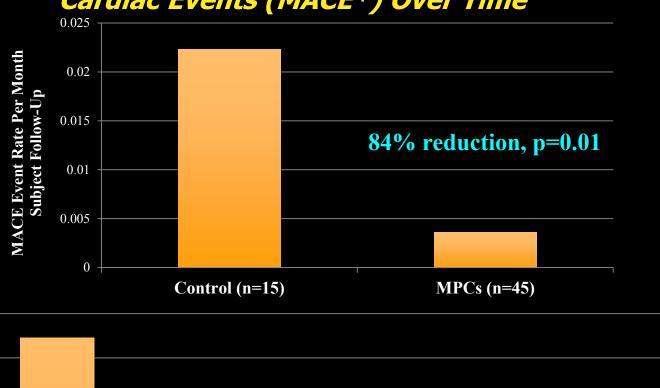
### MPC Treatment Lowers Rate Of Serious Adverse Cardiac Events Over Time

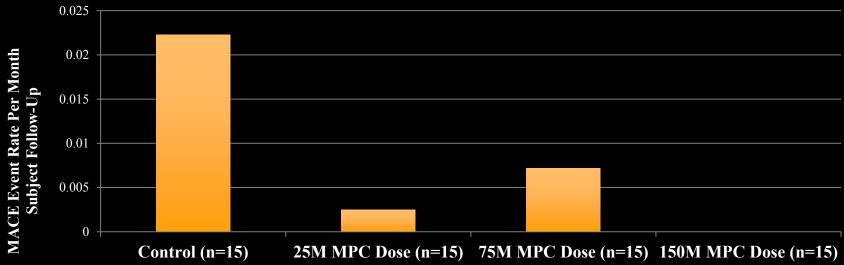


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MPC treated (n=45) followed for total 827.6 person-months Controls (n=15) followed for total 268.5 person-months

## MPC Treatment Lowers Rate Of Major Adverse Cardiac Events (MACE\*) Over Time

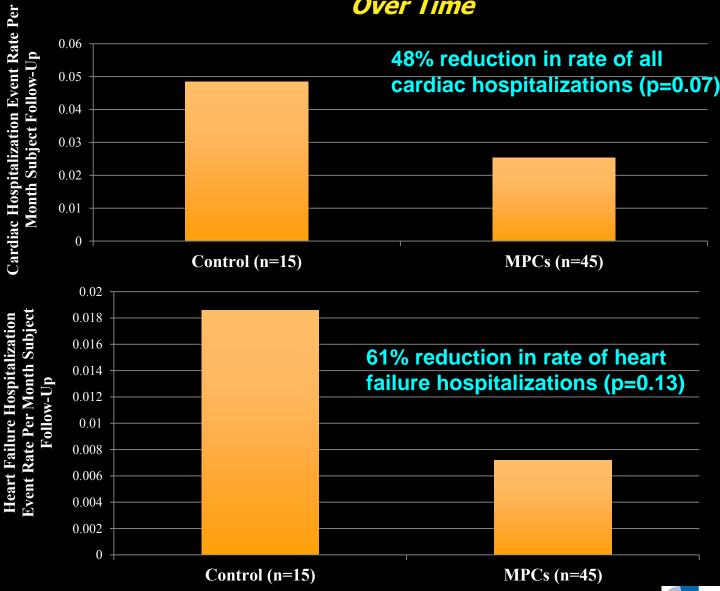




• MACE defined as composite of MI, revascularization, or cardiac death MPC treated (n=45) followed for total 827.6 person-months Controls (n=15) followed for total 268.5 person-months



### MPC Treatment Lowers Rate Of All Cardiac-Related And Heart Failure Hospitalizations Over Time



MPC treated (n=45) followed for total 827.6 person-months Controls (n=15) followed for total 268.5 person-months



### **Conclusions**

### **Safety and Efficacy:**

- Intra-myocardial injection of allogeneic MPC is safe in patients with advanced congestive heart failure
- No cell-related adverse events seen at any dose tested
- Interim analyses indicate that MPC treatment significantly reduces rate of major adverse cardiac events, cardiac-related hospitalization, and mortality over time
- Lowest MPC dose is at least as effective as higher doses tested
- Hard end-points achieved to date form basis for the key primary end-points for FDA Phase 3 trial in heart failure patients





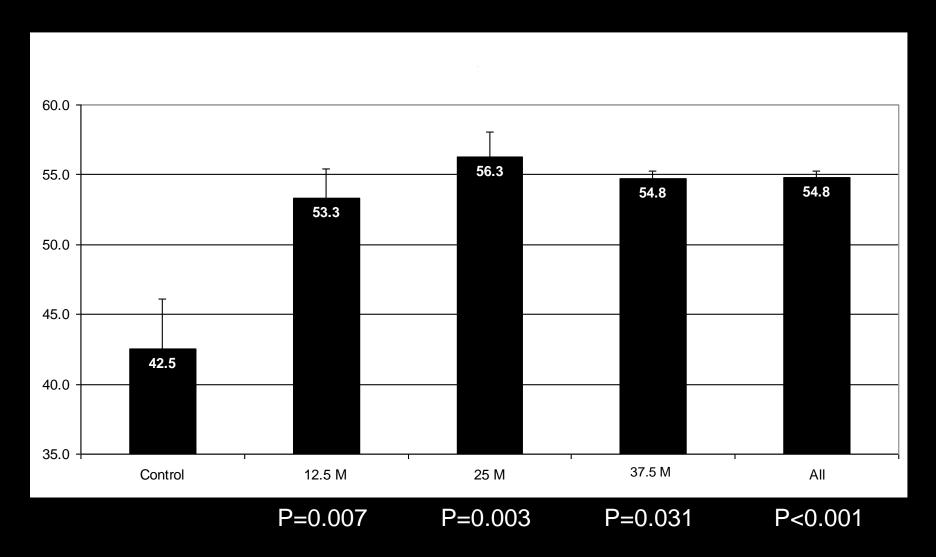
# Intra Coronary Allogeneic Mesenchymal Progenitor Cell (MPC) Transplantation In Myocardial Infarction in Sheep

# Intra-Coronary Infusion Of Allogeneic Sheep MPC In Acute Myocardial Infarction

- A total of 66 sheep
- Balloon occlusion 90 minutes / reperfusion (analogous to angioplasty + stent)
- 30 animals survived the heart attack procedure and were included in analysis
  - 10 control
  - 7 received 12.5M MPC
  - 7 received 25M MPC
  - 6 received 37.5M MPC
- Normal TIMI 3 flow in all animals post cells (i.e. no reperfusion problems)
- 4, 8 week analyses of ejection fraction, left ventricular volumes
- At sacrifice, histology for vascular density, collagen content



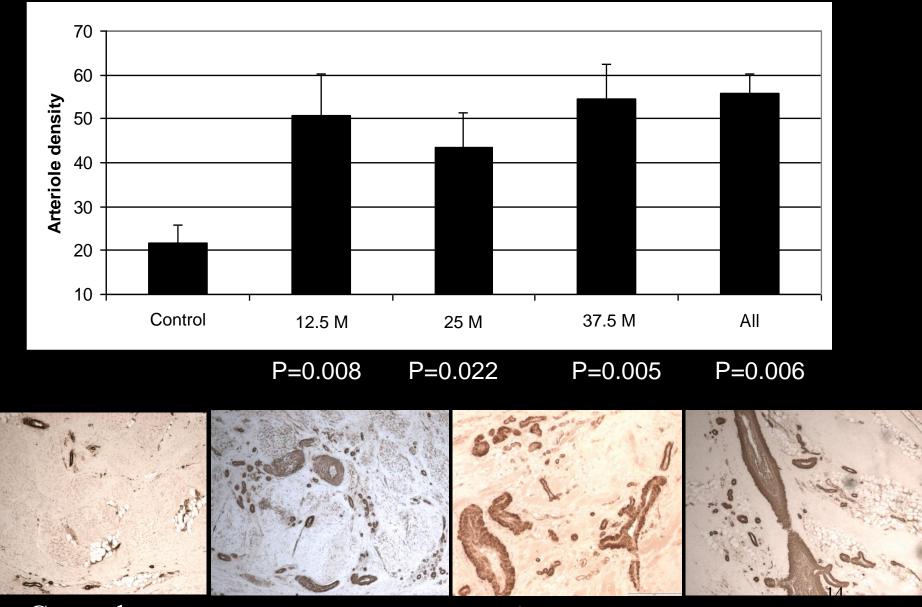
# Ejection Fraction - Global LV function By PV Loop Left Ventricular Ejection Fraction At 8 Week Follow Up





## Arteriolar Density Infarct Zone





Control 12.5 M 25 M 37.5 M

### **Conclusions**

### **Safety and Efficacy:**

- Intra coronary allogeneic MPC infusion is safe and feasible in a large animal model of AMI and reperfusion
- No cell-related flow or arrhythmogenic effects seen
- MPCs significantly improve EF and ventricular volumes
- MPCs induce arteriogenesis
- MPCs reduce scarring and collagen replacement of heart muscle





## Allogeneic MPCs Used As A Feeder Layer To Expand Cord Blood Hematopoietic Stem Cells For Transplantation

# Results Of Double Cord Transplants Using MPC Expansion

- 25 patients transplanted, under IND protocol
- Avg. age 40 yrs, all with myeloablative regimen for disease relapse
- Cord CD34+ HSC expanded >44-fold by co-culture with allogeneic MPC
- Median time to neutrophil engraftment 15 days
- Median time to platelet engraftment 54 days
- 16% (4/25) patients have Grade III/IV GVHD
- 80% (20/25) patients engrafted neutrophils by day 26 and survived at 100 days

(compared with 46% non-expanded double cord, Brunstein et al Blood 2010)



#### Comparison Of Angioblast Results With CIBMTR Historical Controls 18

In 2008 and 2009 there were 596 non-expanded double cord transplants registered with the CIBMTR across 79 transplant centers.

(300 with myeloablative conditioning regimens)

	Controls	MPC
Median neutrophil engraftment time	26 days	15 days
Median platelet engraftment time	>120 days	54 days
Neutrophil engraftment <26 days + alive at day 100	46%	84%

