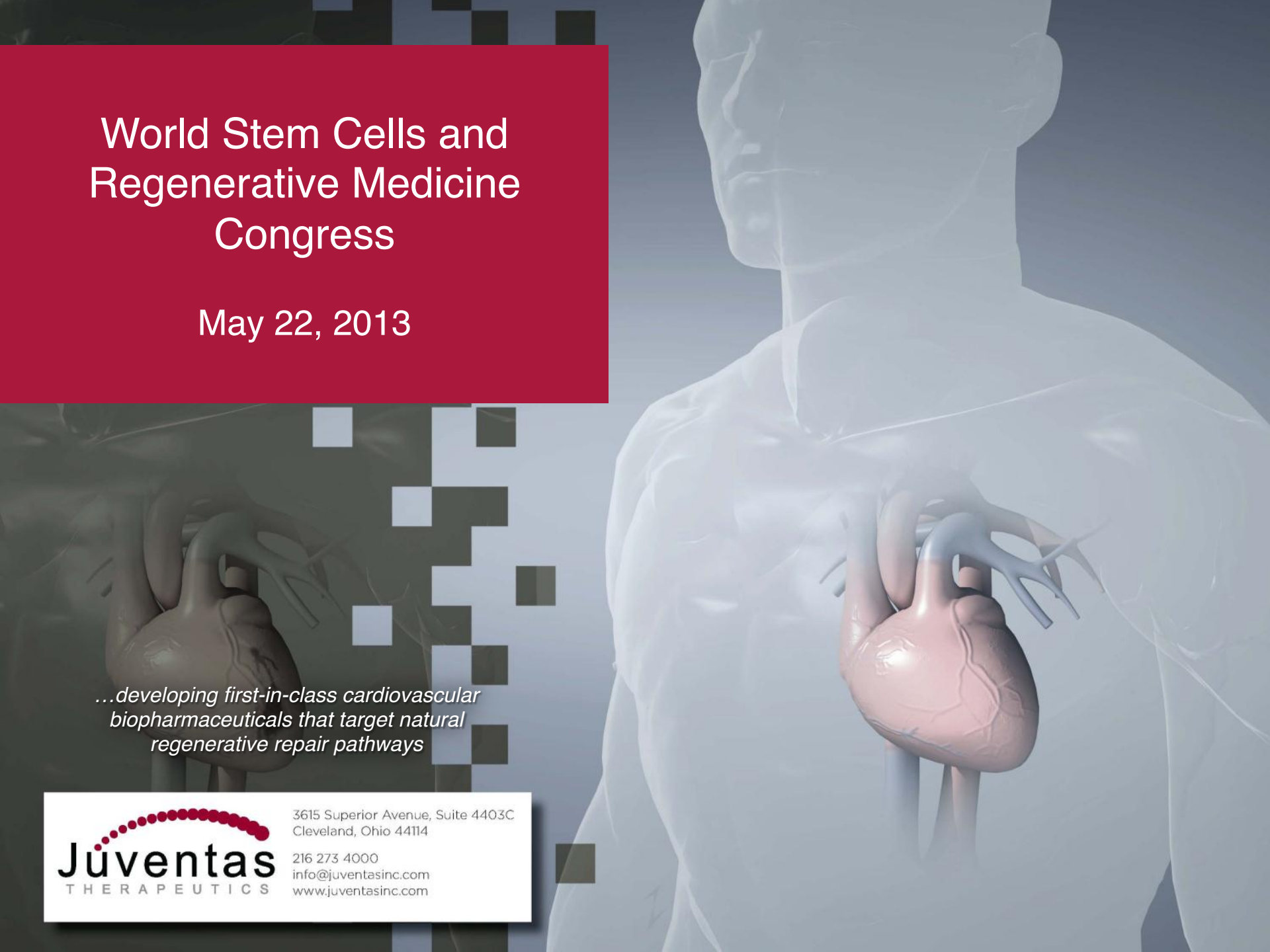
World Stem Cells and Regenerative Medicine Congress

May 22, 2013



…developing first-in-class cardiovascular biopharmaceuticals that target natural regenerative repair pathways

Juventas THERAPEUTICS

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Executive Summary

Venture-backed, clinical-stage biotechnology company developing first-in-class therapies for cardiovascular disease

Applying factor-based strategy to activate or enhance endogenous stem cell based repair pathways

Lead product **JVS-100** encodes Stromal Cell Derived Factor 1 (SDF-1) currently the subject of Phase II clinical trials in heart failure and critical limb ischemia

Developing **JVS-200** which encodes monocyte-specific chemokine 3 (MCP3) as an adjunct to enhance cell therapy strategies

The Hypothesis

Stem cell based repair of ischemic tissue in mammals is a natural process but clinically inefficient due to dysregulation or short term expression of key molecular factors

Reintroducing molecular factors that regulate stem cell based repair at a time remote from injury may drive tissue repair without the cost and complexity of cell therapy

JVS-100 (SDF-1)

Activating endogenous stem-cell based repair pathways

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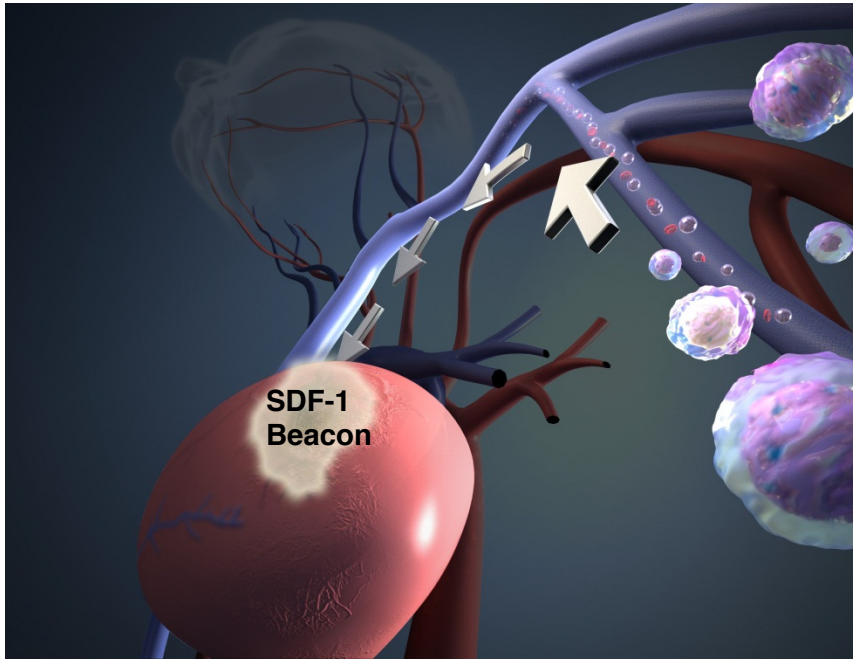
SDF-1: A Natural Repair Signal

Recruits endogenous stem cells to damaged organ, promoting new blood vessel formation, and preventing on-going cell death

Naturally upregulated in response to tissue injury but expression is too short-lived to provide meaningful benefit

More than 100 publications describing SDF-1 biology and mechanisms of action associated with tissue repair



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The Juventas Solution: JVS-100

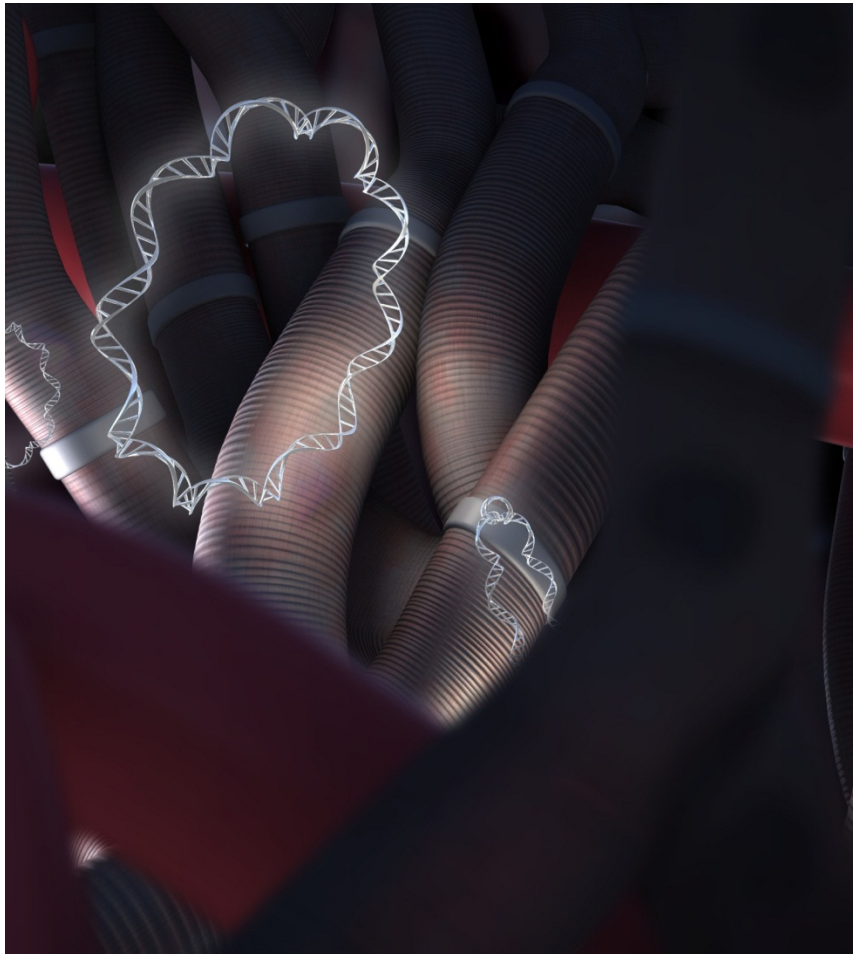
JVS-100 is a non-viral, DNA plasmid encoding SDF-1

Yields therapeutically relevant protein expression for ~17 days

Direct delivery to damaged tissue

Highly scalable manufacturing

Stable for at least 24 months at -20°C



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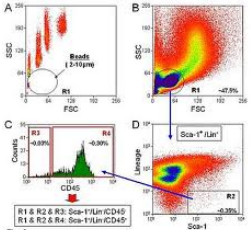
Transforming Process Into Product

Cell Therapy

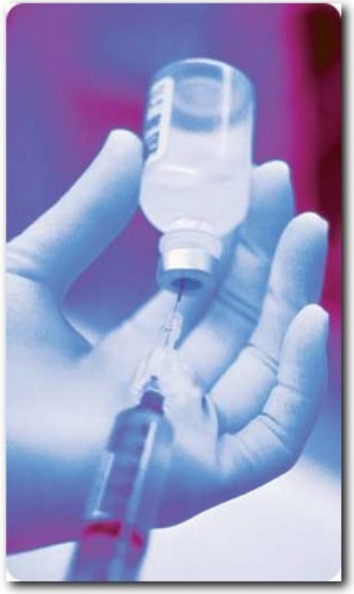
Bone Marrow Harvest

Cell Selection



Cell Propagation

JVS-100



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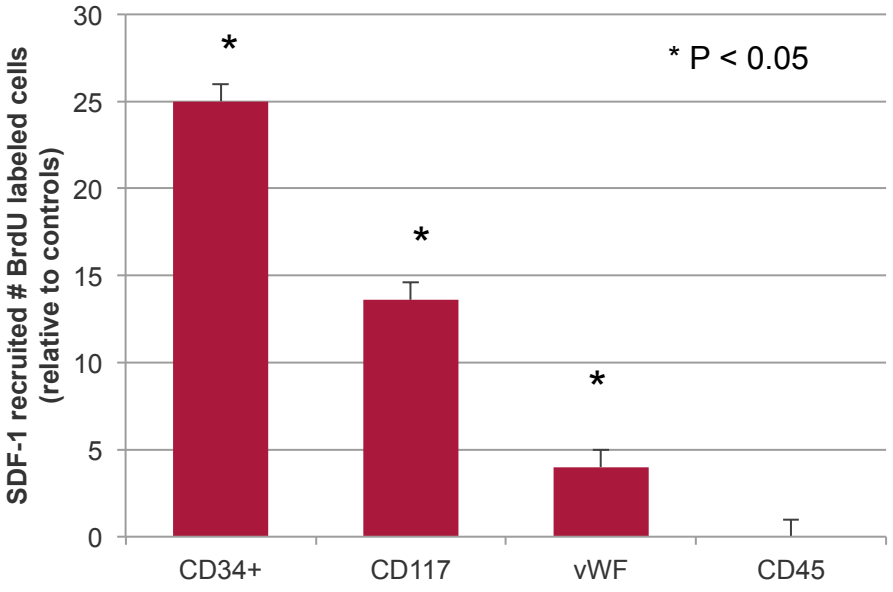
SDF-1 recruits CD34+ and CD117+ cells to damaged tissue

- Chronic Heart Failure (CHF) rat model treated 2 months post-myocardial infarction
- SDF-1 recruits endogenous HSCs to damaged tissue
- Recruitment demonstrated in heart, brain, peripheral vasculature, kidney, and epithelium



Chronic Heart Failure Rat Model

Askari et al. Lancet. 2003 Aug 30;362(9385):697-703.

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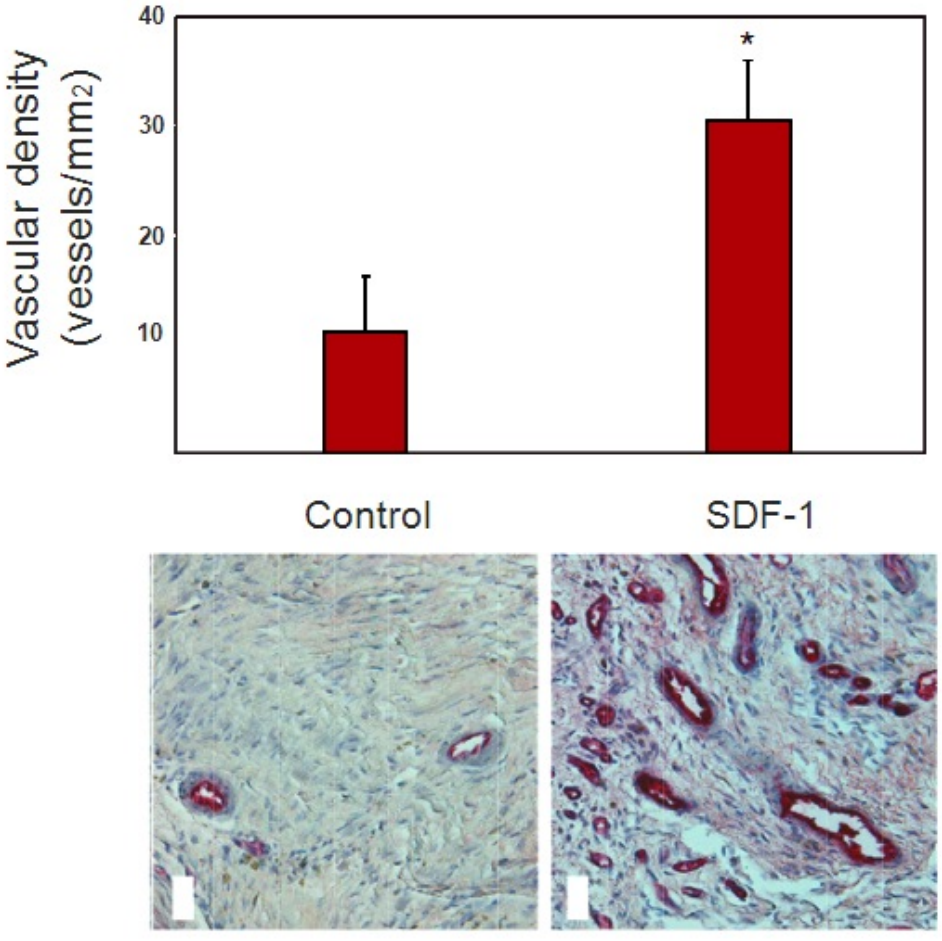
SDF-1 is Vasculogenic



Chronic Heart Failure Rat Model

Askari et al. Lancet. 2003 Aug 30;362(9385):697-703.

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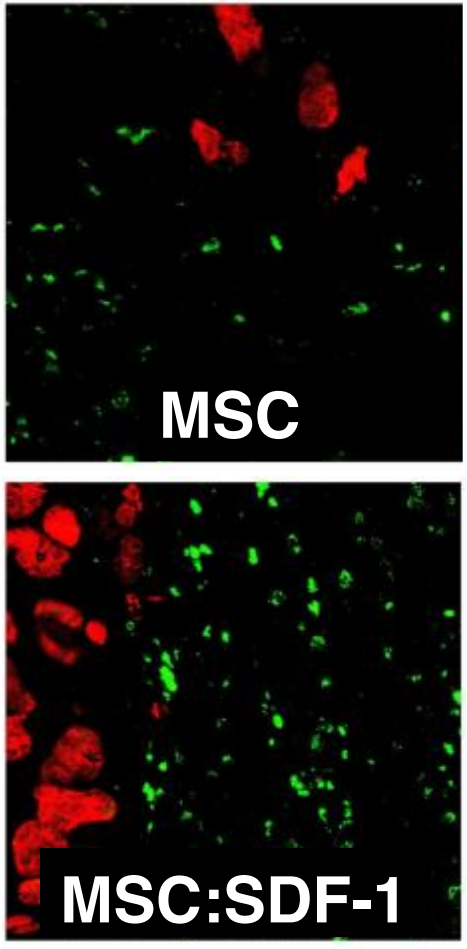
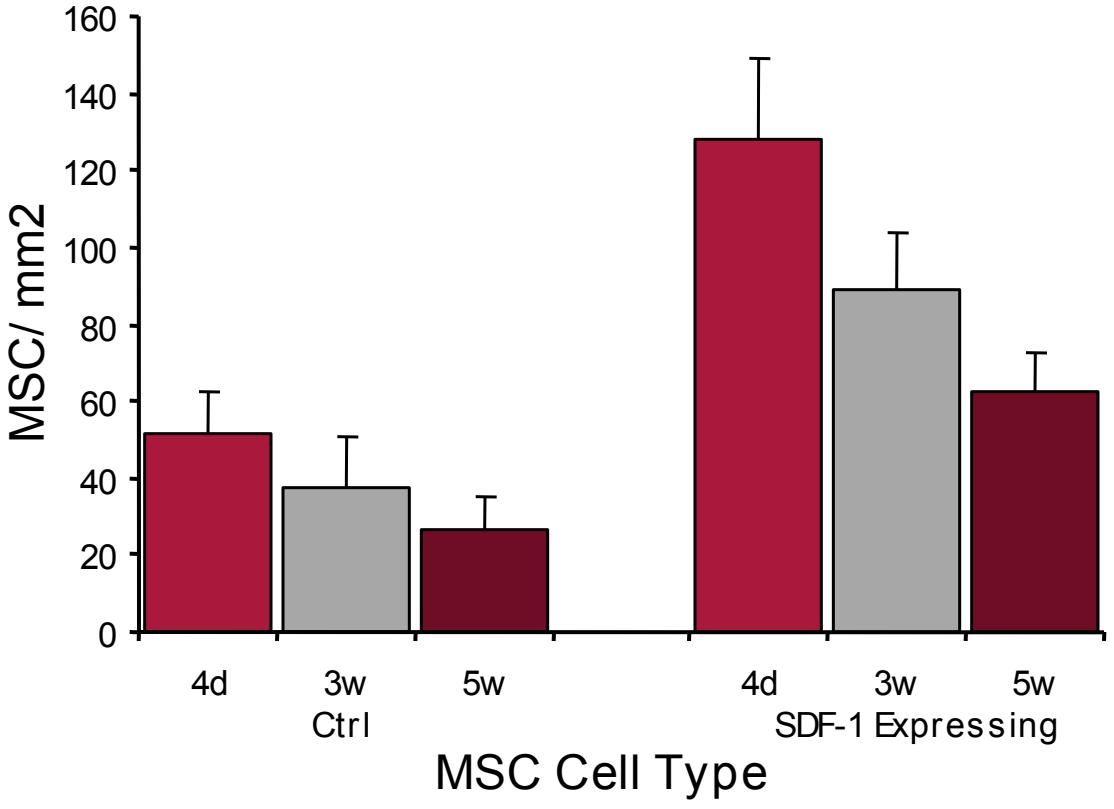
SDF-1 expression promotes MSC survival





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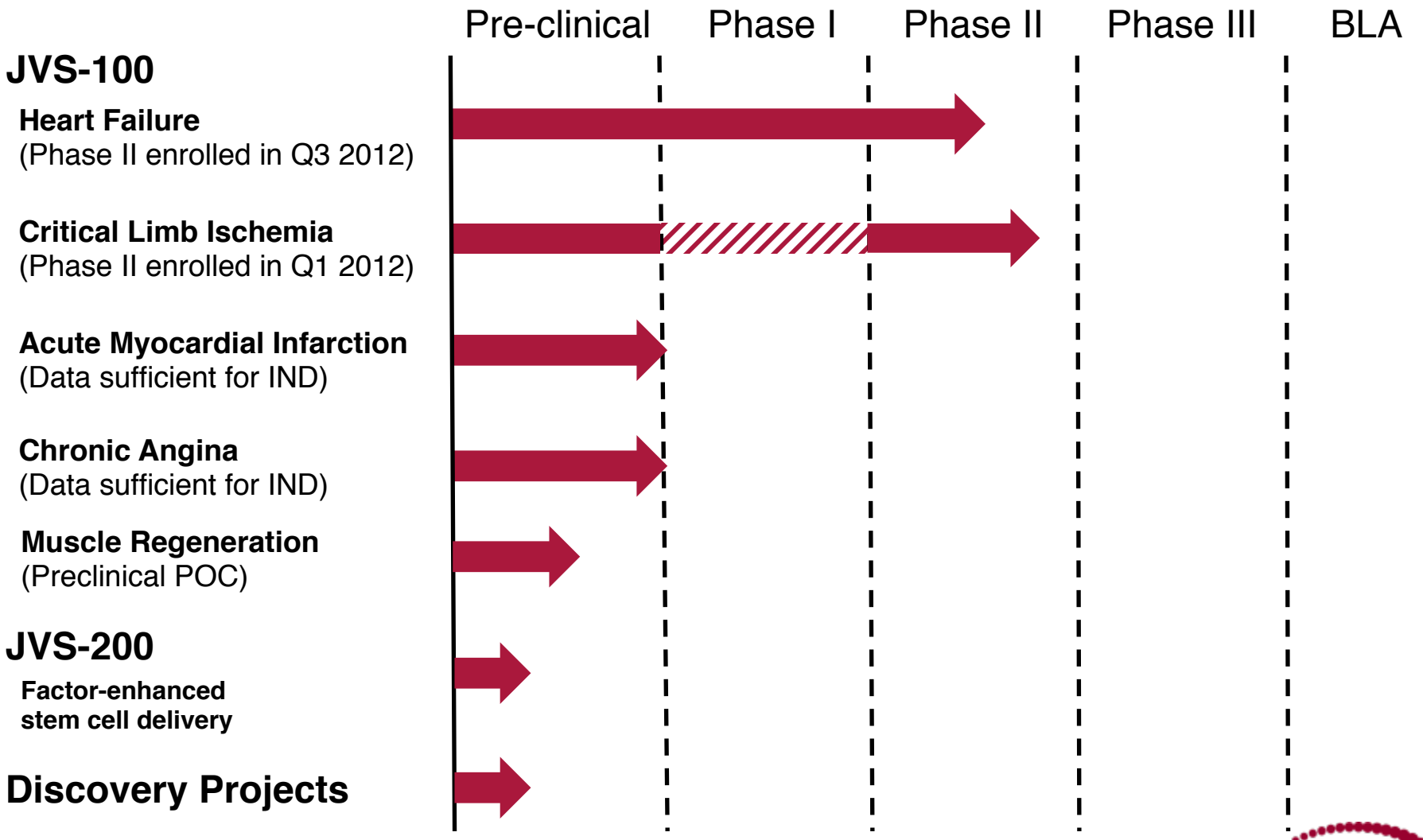
Juventas Product Pipeline



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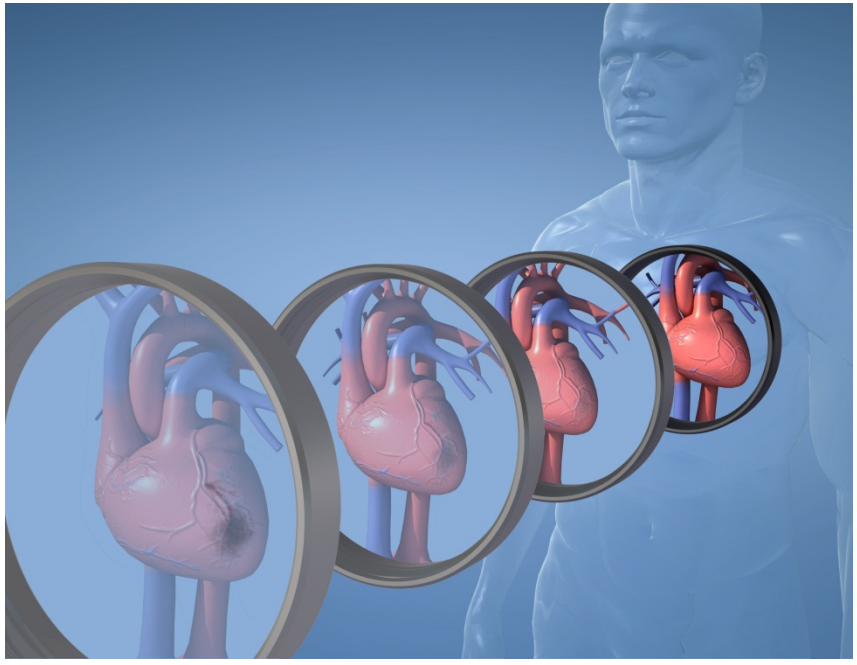
Symptomatic Ischemic Cardiomyopathy

Heart failure and cardiac dysfunction that occurs in response to prior myocardial infarction

> 5.7 million people with heart failure in the United States with an additional 670,000 new patients affected each year

Target clinical population:

3 million people suffer from ischemic cardiomyopathy in the United States



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Phase I HF Trial Summary

published in Circulation Research (March 2013)

17-patient, open label, dose escalation trial in symptomatic ischemic heart failure population.

One-time treatment with 5, 15 or 30 mg

Delivery via endoventricular injection catheter (*Biocardia Helix*), 15 injections of 1 mL each

Participating Centers: Cardiology P.C (Birmingham, AL); Columbia (New York, NY; Northwestern (Chicago, IL); Rush Medical (Chicago, IL)

Patient Profile	
Age (yr)	66 ± 9
Time since last MI (yr)	7 ± 7
Gender (% Male)	71%
NYHA Class III	94%
6 Min. Walk (m)	290 ± 91
QoL Score	54 ± 21
LVESV (ml)	109 ± 35
LVEF (%)	32.5±5.5
Ischemic CM (%)	100%
SDS	4 ± 5

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Phase I HF Trial Summary

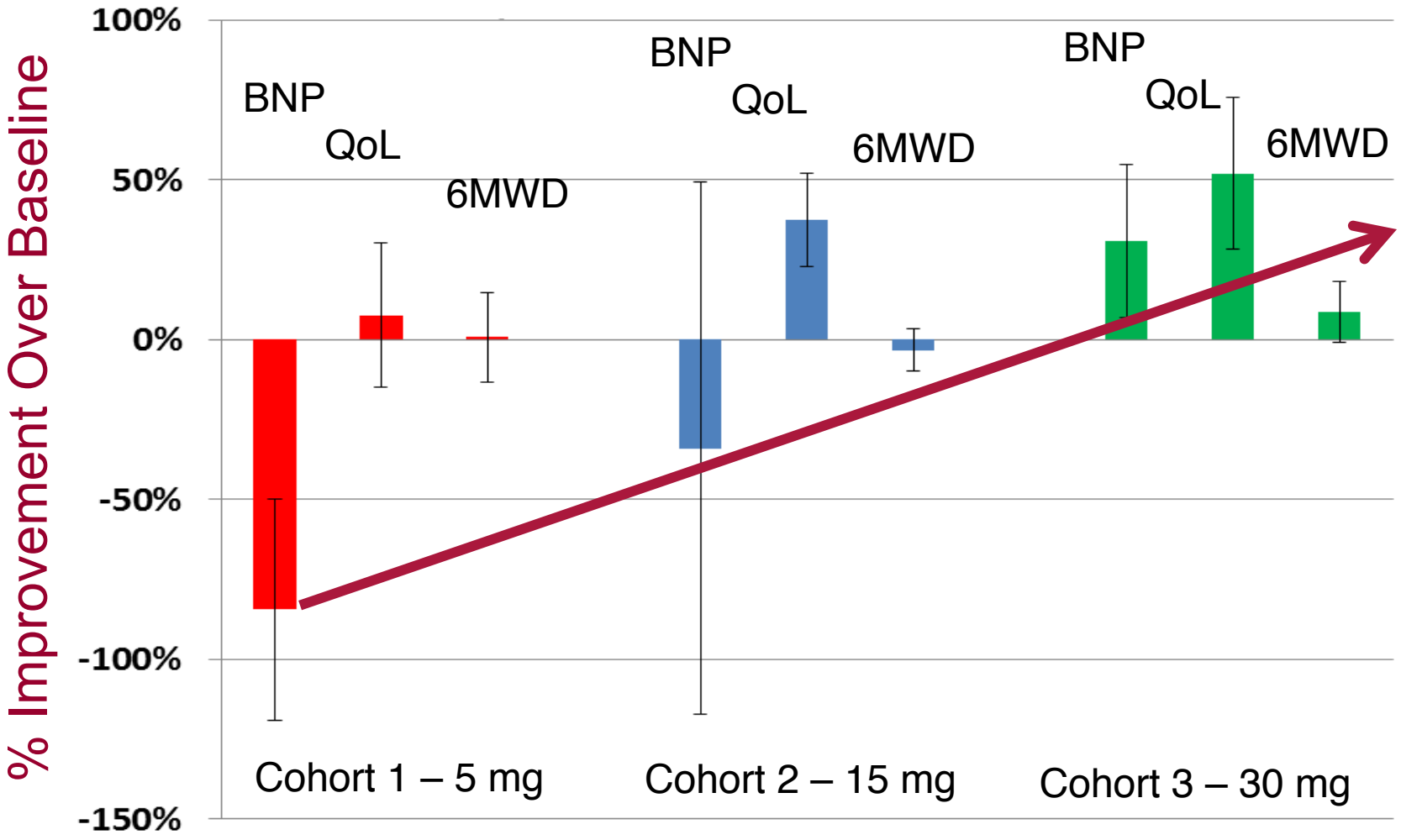
Safety

- Primary safety endpoint met
- No drug related SAEs reported
- 15 of the 17 patients surviving at least 18 months post-treatment. Two deaths related to underlying disease.

Efficacy

- Dose dependent improvements in QOL, 6MWD and BNP
- Dose dependent stabilization LVEF and LVESV
- Improved NYHA class in >50% of study subjects at 12 months

Phase I Data Summary: 12 months



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Phase II Trial: STOP-HF

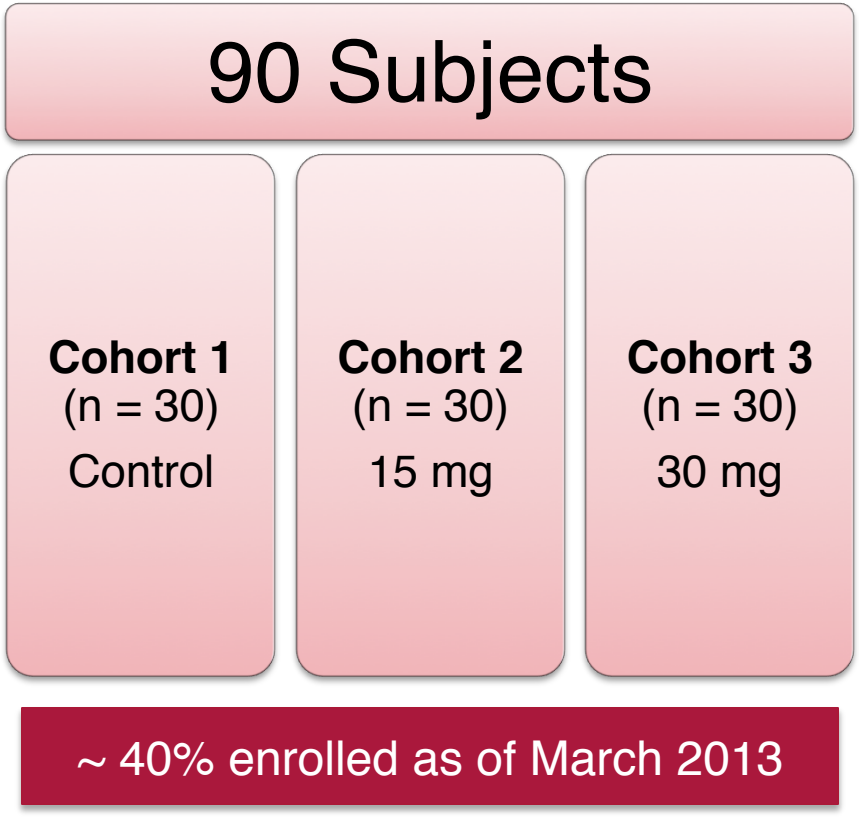
Placebo-controlled, randomized, double-blinded design

Inclusion/Exclusion equivalent to Phase I trial design

Primary efficacy endpoint will be a composite of 6MWd, QoL and NYHA with collection of mortality and hospitalization data

16 US sites

Data targeted Q1 2014



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Critical Limb Ischemia (CLI)

Advanced stages of peripheral vascular disease

- PVD affects more than 18 million people in the United States

Estimated 3,000,000 diagnosed with CLI in the United States

- ~200,000 new cases annually

35% of patients undergo amputation within 12 months of diagnosis

CLI is the leading cause of amputation outside of military related trauma

Each amputation costs the healthcare system approximately $500,000

Phase IIa Trial: STOP-CLI

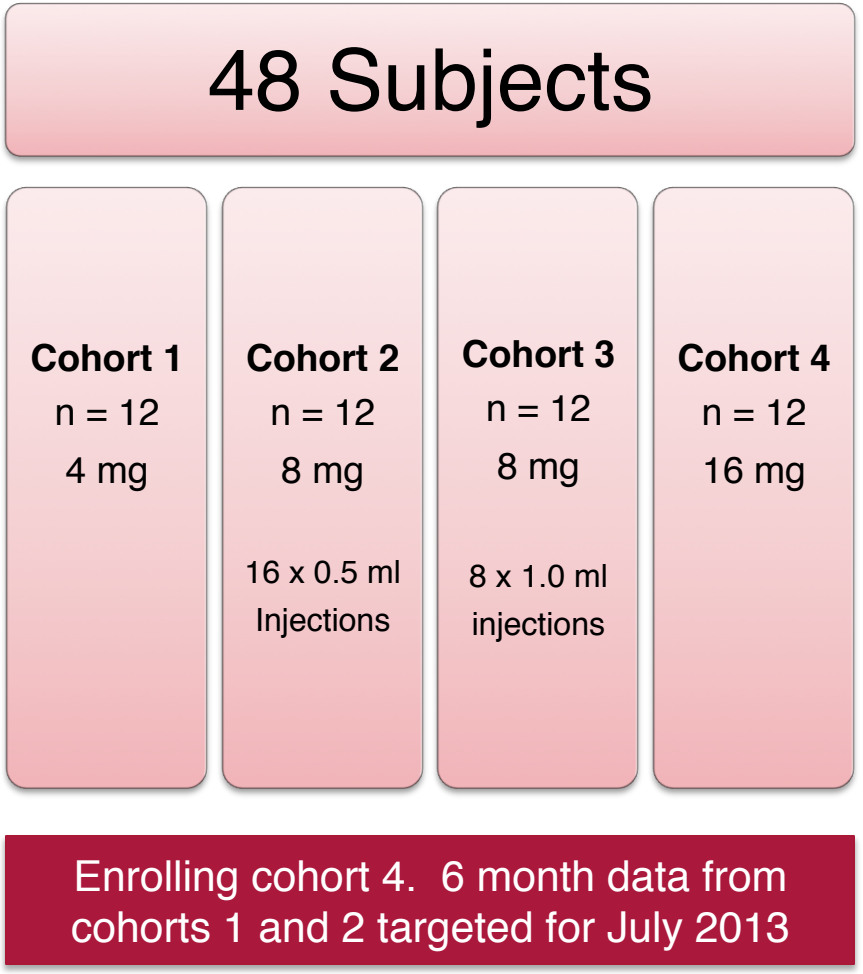
Double-blinded, placebo-controlled, dose-escalation trial design

JVS-100 injected into ischemic limb

Inclusion criteria: Rutherford 4 or 5, ABI≤0.4, ankle pressure <70 mmHg, toe pressure <50 mmHg

Enrolling at 7 sites in US and India

Efficacy endpoints include amputation, wound closure, and reduction in pain medication



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JVS-200 (MCP3)

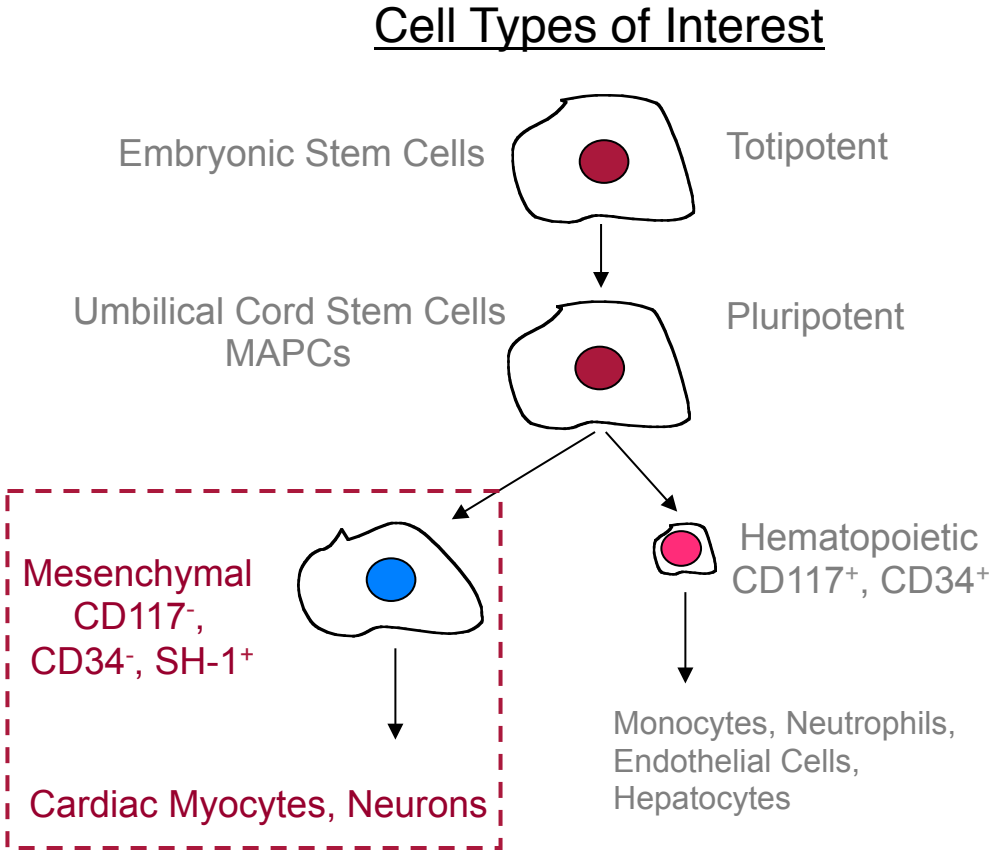
Enhancing autologous and allogeneic cell therapy strategies

MCP3: Monocyte-specific chemokine 3

Monocyte-specific chemokine 3 (MCP3) is a cytokine that homes mesenchymal stem cells

Preclinical myocardial infarction study demonstrated that treatment with MCP3 followed by MSCs significantly improved cell recruitment, retention, and overall therapy efficacy when compared to treatment with MSCs alone*

*S. Schenk, et al. *Stem Cells.* 2007; 25(1):245-251



Juventas Strategy: *Induce MCP3 expression and follow with serial infusion of MSCs to provide superior efficacy profile*

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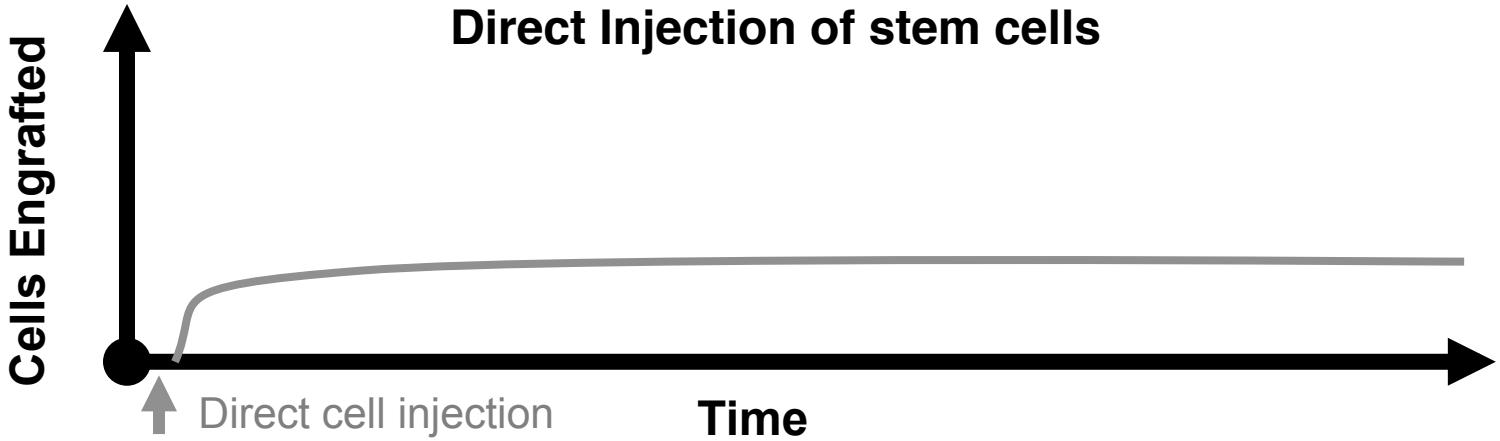
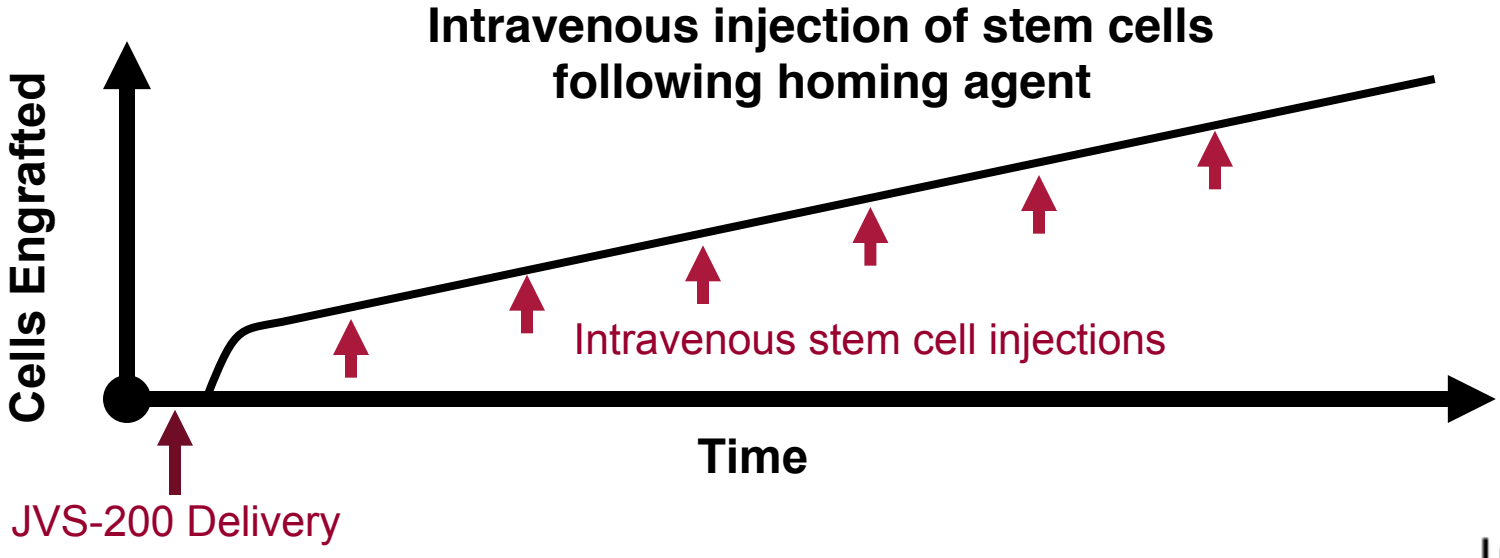
JVS-200 Homing Can Enhance Cell Therapy





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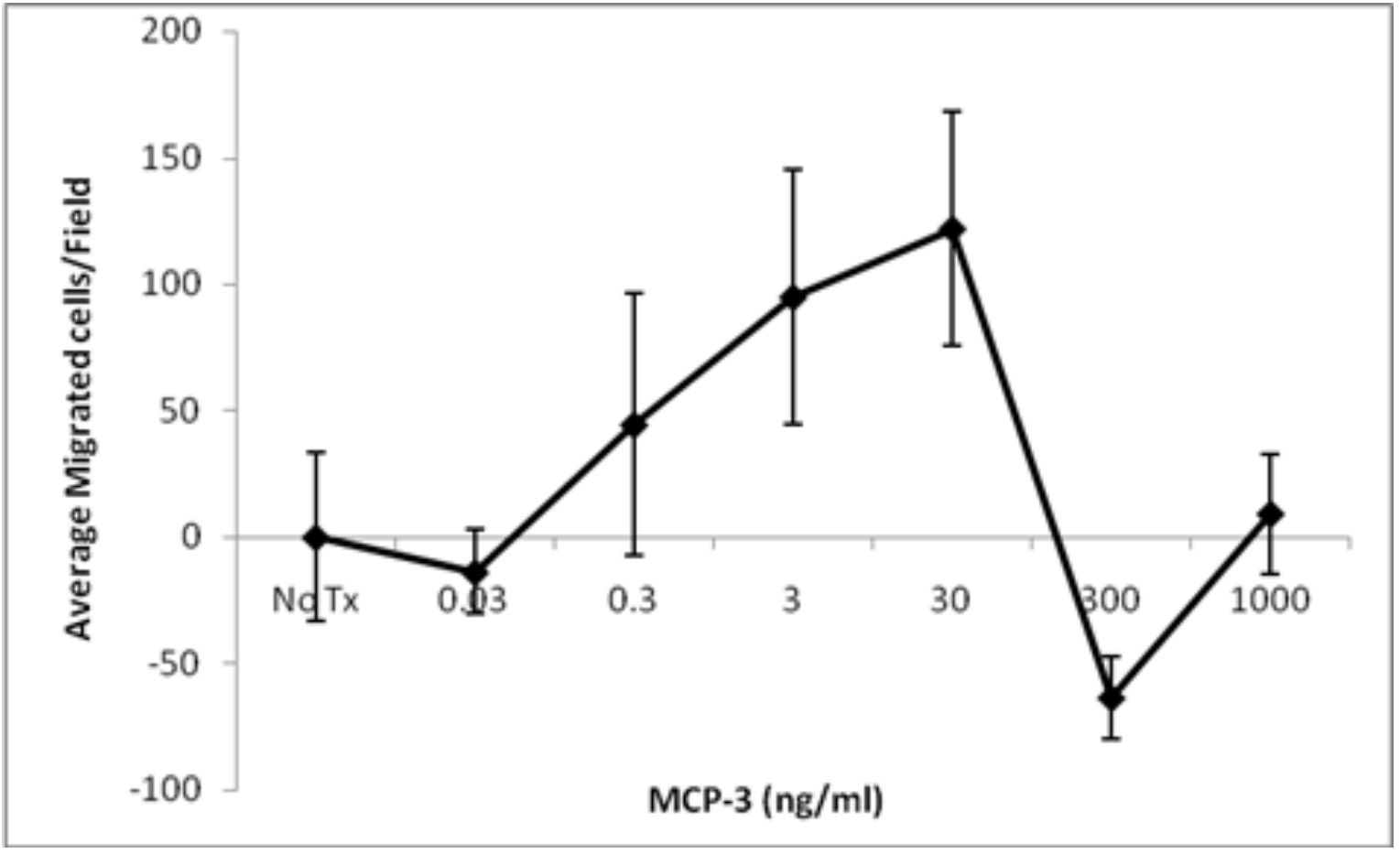
MCSs home in a dose responsive fashion to MCP3



S. Schenk, et al. *Stem Cells*. 2007; 25(1):245-251

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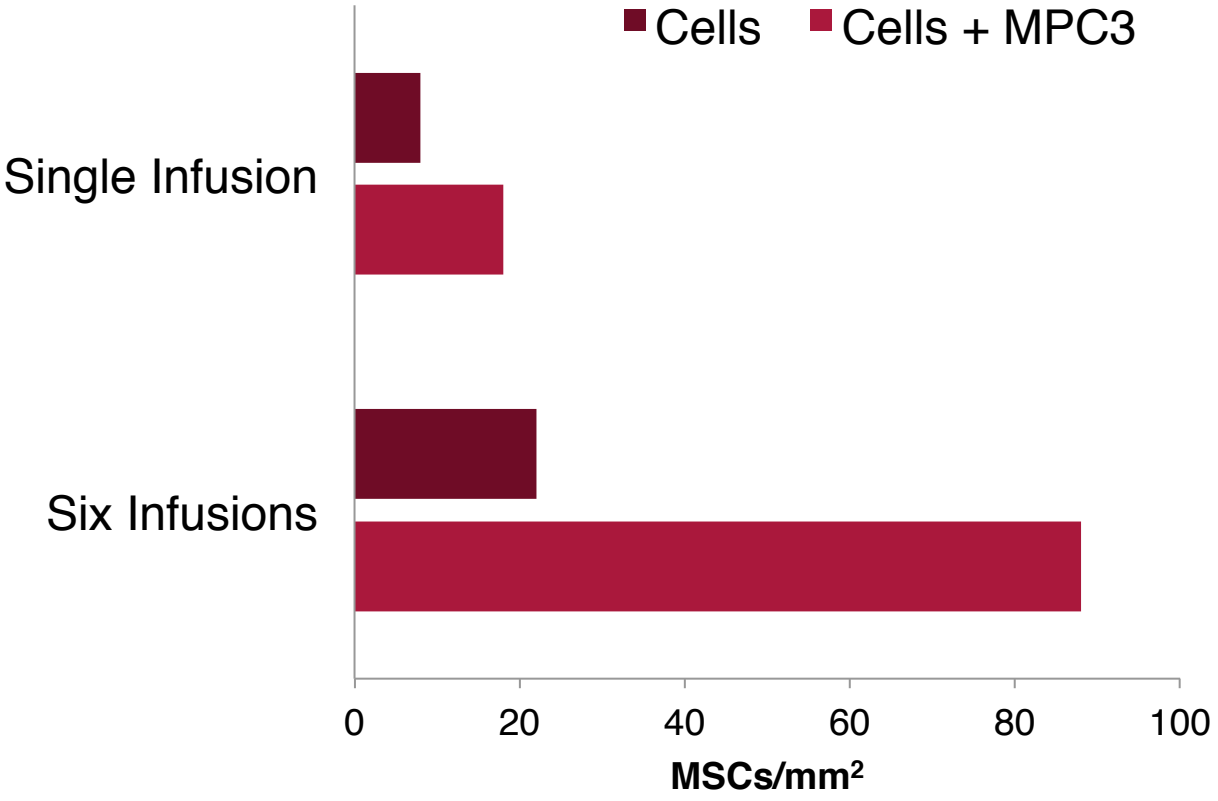
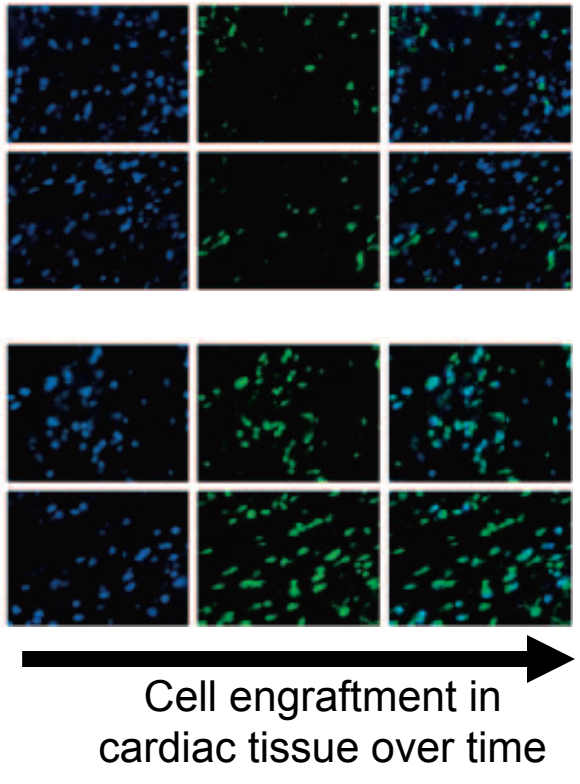
MCP3 Enhances Cell Recruitment and Retention





In vivo cell retention increases in the presence of MCP3 with multiple infusions of MSCs

S. Schenk, et al. *Stem Cells*. 2007; 25(1):245-251

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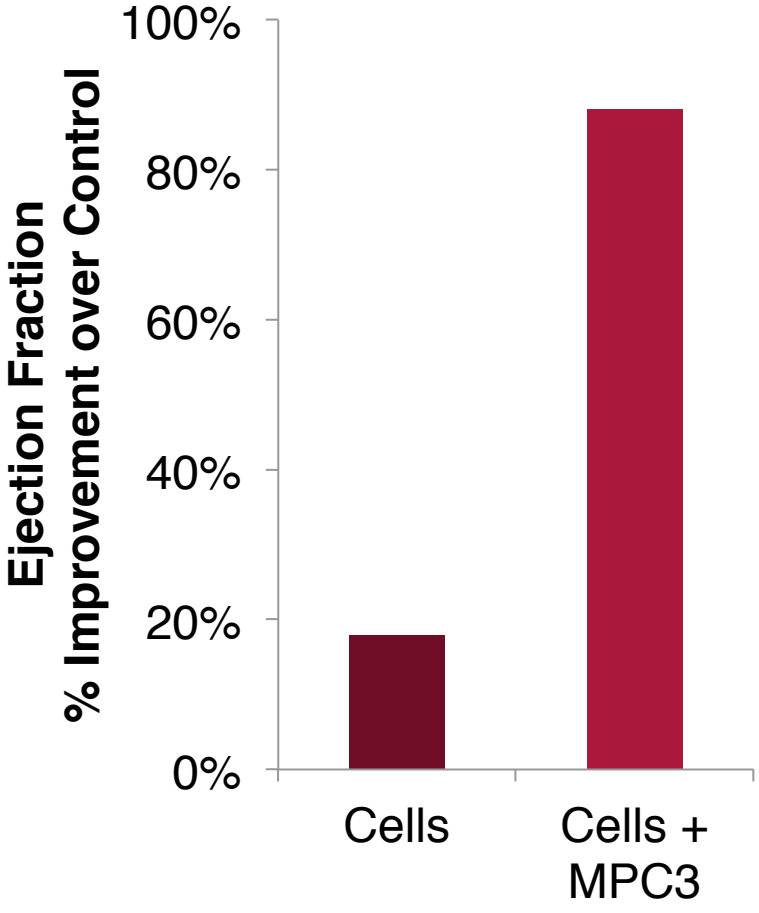
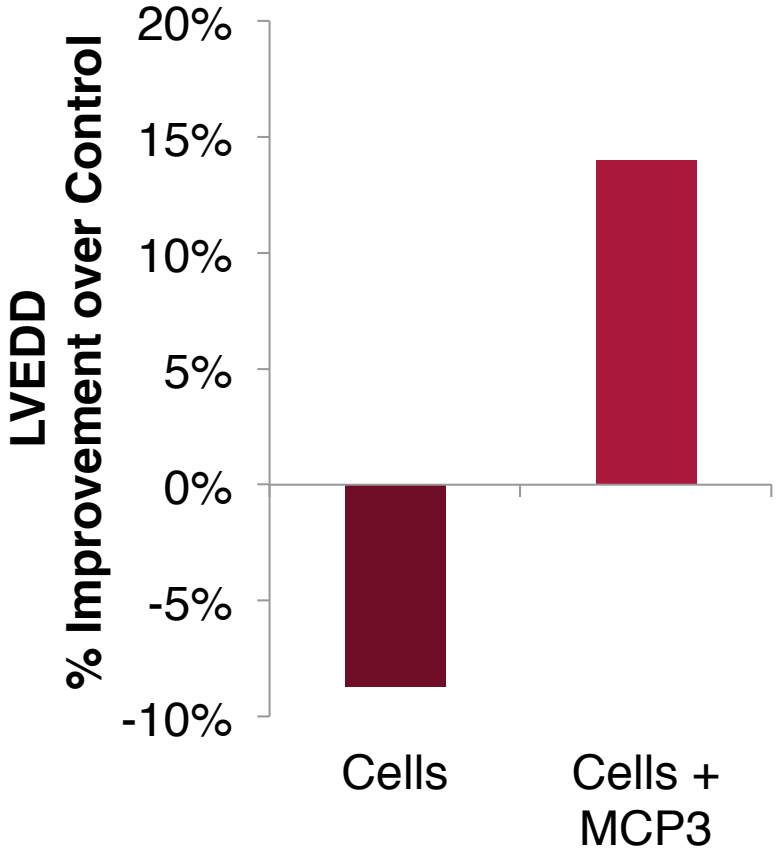
JVS-200 Improves Heart Function

Treatment 1 month post-MI





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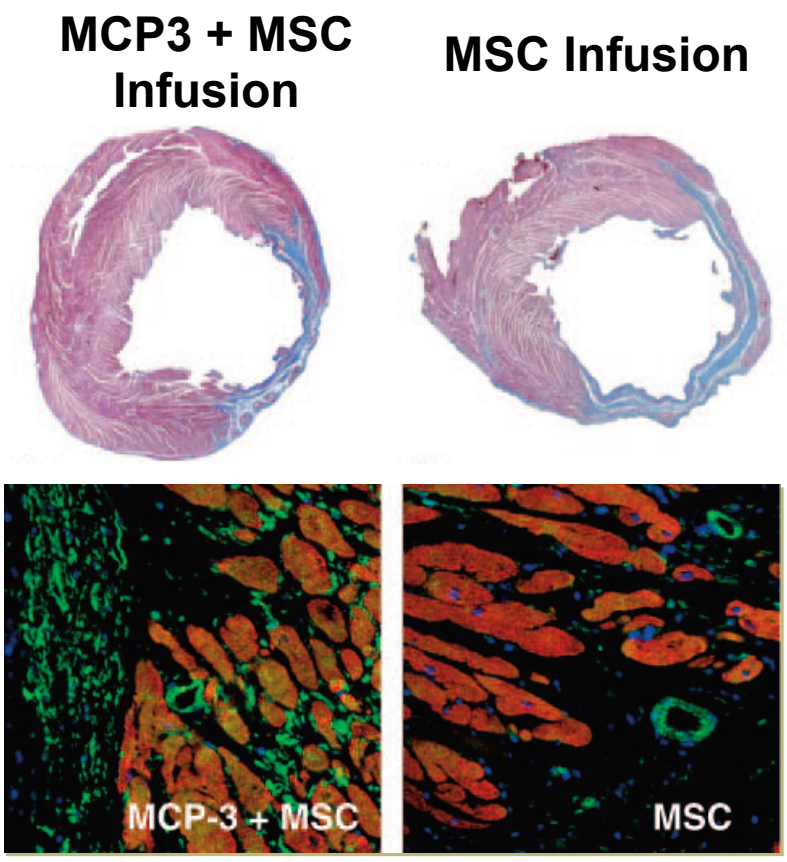
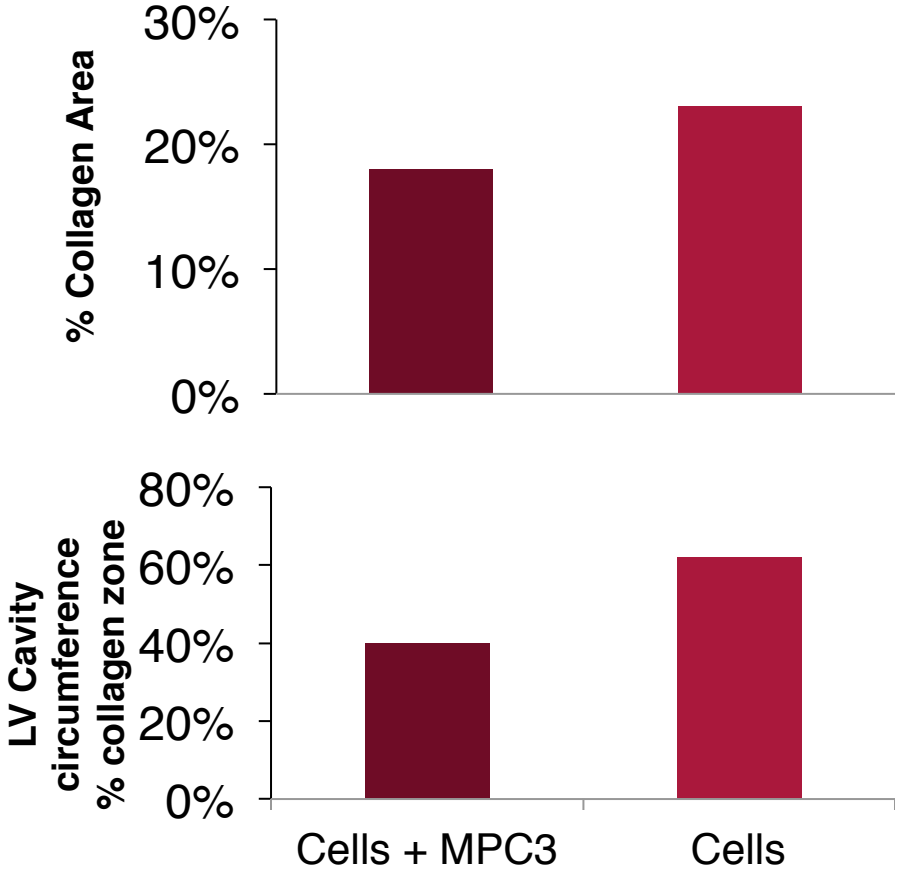
MCP3 Induces Cardiac Remodeling





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MCP3 followed by MSC infusion results in beneficial remodeling and myofibroblast recruitment

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Homing Factor Advantage

Efficacy	Superior efficacy due to the synergistic approach of MCP3 expression with cell infusion
Delivery of MSCs to site of ischemia	MSCs may be dosed multiple times through infusion over 3 weeks after one injection of JVS-200 at site of ischemia
Timing	JVS-200 may be dosed while patient has other procedure (e.g. in cath lab) followed by MSCs delivered by IV after harvesting complete

Working Conclusion

Non-viral gene therapy provides an therapeutic modality that allows for effective and cost-efficient delivery of molecular factors that activate or enhance natural stem cell driven tissue regeneration

JVS-100 activates endogenous stem cell repair pathways providing regenerative medicine benefits without the associated cost and complexity of harvesting, expanding and delivery typically associated with cell therapy

JVS-200 when delivered in conjunction with mesencymal stem cells may enhance efficacy of existing cell therapy strategies and allow for introduction of serial intravenous infusions.