An Innovative Cell-Based Therapy for Ischemic Vascular Disease: Direct Isolation and Infusion of CD31^{+} Cells From Bone Marrow

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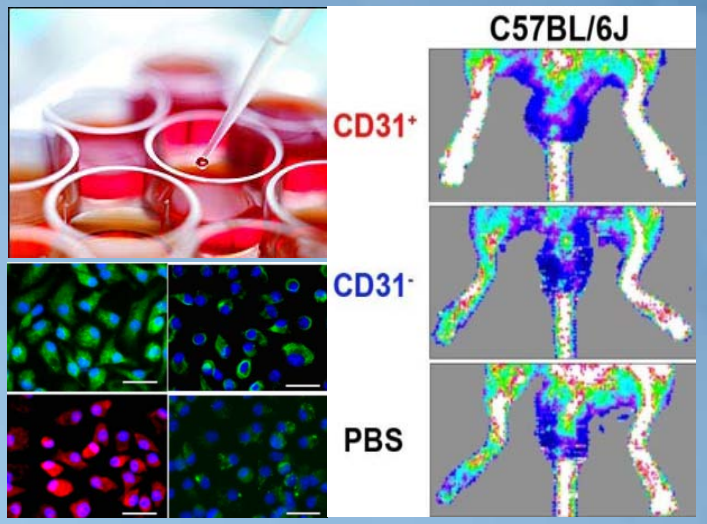


Value Proposition

- Highly angiogenic/vasculogenic bone marrow-derived cells for the treatment of ischemic vascular diseases
- Novel use of known cell surface marker: CD31
- Yields therapeutic quantities of cells enabling direct infusion back into the patient
- Potentially reduced toxicity
- Device-based business model
- Unique, disruptive cell-based therapy

Background

- Formation of new blood vessels
  - Vasculogenesis: de novo development of blood vessels from endothelial progenitor cells (EPCs)
  - Angiogenesis: reorganization of endothelial cells from preexisting blood vessels
- EPCs found in peripheral blood and bone marrow (BM)
- EPCs isolated via physical properties (adherence, morphology, etc.)

Unmet need: No specific marker exists for identifying highly enriched angio-vasculogenic cells from BM

CD31: A Marker to Identify Highly Angio-Vasculogenic Cells From Bone Marrow

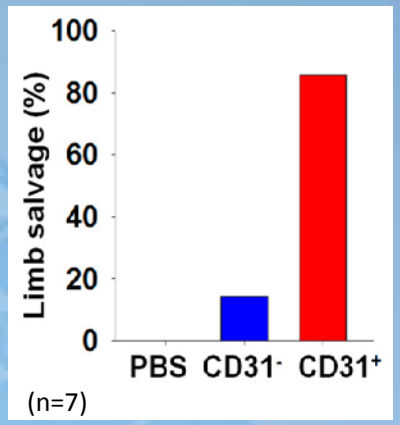
EMORY

Nude Mice

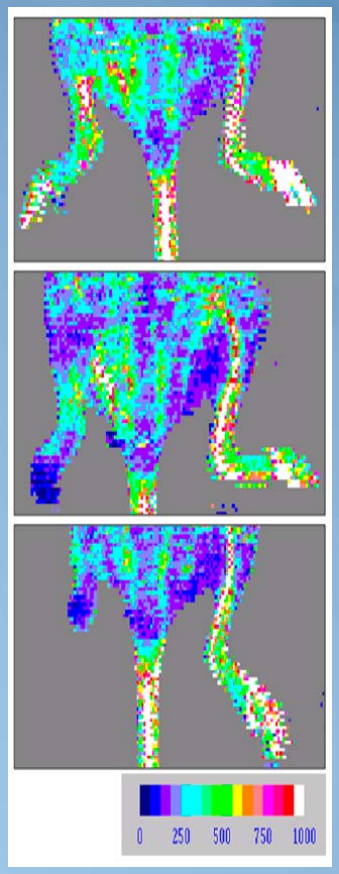
CD31+

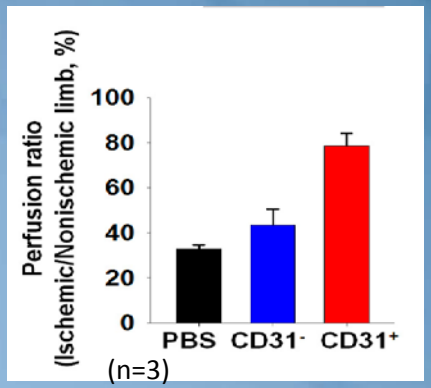
CD31-

PBS

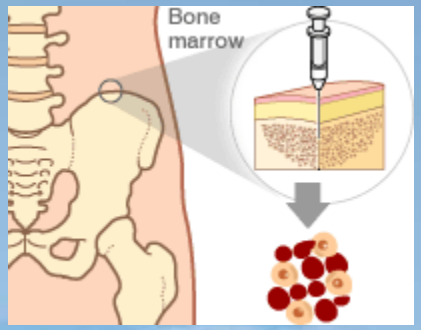


Doppler Perfusion

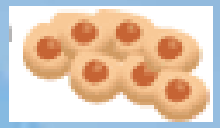




Proposed Business Model: Autologous CD31^{+} Cell Therapy



Bone marrow removed from patient



CD31^{+} cells isolated with extractor onsite

Direct infusion of CD31^{+} cells into patient

Competitive Analysis

Technology Owner	Production of Cells	Business Model	Comments
Emory	Infused Directly	Device	Viable business model, potentially reduced toxicity
Aastrom Biosciences	Expanded prior to infusion	Service	Clinical trial halted due to adverse events
Aldagen	Infused directly	Service	Heterogeneous cell population-Dangerous?
Osiris Therapeutics	Expanded prior to infusion	Therapeutic product	One marketed product, two others in advanced clinical trials-Dangerous?
Various ES Companies	Differentiated and expanded prior to infusion	Therapeutic product	Earlier stage of development, ethical issues

Potential Indications

- Critical Limb Ischemia
  - Affects at least 600,000 people in the U.S.
  - Leads to over 160,000 major limb amputations/year in U.S.
- End-Stage Ischemic Heart Disease
  - 3,000,000 persons in the U.S. suffer from congestive heart failure due to coronary artery disease
  - 50% five-year survival rate
- Post-Acute Myocardial Infarction
  - Approximately 700,000 individuals in the U.S. each year experience their first heart attack

Intellectual Property

- "Therapeutic Use of CD31 Expressing Cells"
  - U.S. national stage utility application pending (filed 4/2009)
  - Compositions and Methods of Use
- Potential to strengthen IP position through development of $CD31^{+}$ cell extractor device

Competitive Advantage

- Proprietary marker (CD31) for identifying highly angio-vasculogenic cells from bone marrow
- High cell yield enables direct infusion
- May overcome toxicity (expansion/heterogeneity)
- Utilizes off-the-shelf technology (antibody conjugated magnetic beads, FACS)
- Sustainable business model
  - Cells prepared onsite via device with disposables
  - Target initial indications with severe unmet need

Future Plans

- Demonstrate proof-of-principle in cardiac ischemia animal model
- Perform additional experiments to examine toxicity and safety of $CD31^{+}$ cells
- Commercialization plan: start-up vs. established co.
- If start-up route, obtain seed funding to develop cell extraction device