## UNITED STATES SECURITIES AND EXCHANGE COMMISSION Washington, D.C. 20549

#### FORM 8-K

### CURRENT REPORT Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): September 8, 2014

#### NEOSTEM, INC.

(Exact Name of Registrant as Specified in Charter)

Delaware (State or Other Jurisdiction of Incorporation) 001-33650 (Commission File Number) 22-2343568 (IRS Employer Identification No.)

420 Lexington Avenue, Suite 350, New York, New York 10170 (Address of Principal Executive Offices)(Zip Code)

#### (212) 584-4180

Registrant's Telephone Number

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

#### Item 7.01 Regulation FD Disclosure.

NeoStem, Inc. intends, from time to time, to present and/or distribute to the investment community and utilize at various industry and other conferences a slide presentation. The slide presentation is accessible on NeoStem's website at www.neostem.com and is attached hereto as Exhibit 99.1. NeoStem undertakes no obligation to update, supplement or amend the materials attached hereto as Exhibit 99.1.

In accordance with General Instruction B.2 of Form 8-K, the information in this Item 7.01 of this Current Report on Form 8-K, including Exhibit 99.1, shall not be deemed "filed" for the purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Exchange Act or the Securities Act of 1933, as amended, except as shall be expressly set forth by reference in such a filing.

#### Forward Looking Statements

This Current Report on Form 8-K, including Exhibit 99.1 hereto, contains "forward-looking" statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are typically preceded by words such as "believes," "expects," "anticipates," "intends," "will," "may," "should," or similar expressions, although some forward-looking statements are expressed differently. Forward-looking statements represent the Company's management's judgment regarding future events. Although the Company believes that the expectations reflected in such forward-looking statements are reasonable, the Company can give no assurance that such expectations will prove to be correct. All statement other than statements of historical fact included in the Current Report on Form 8-K are forward-looking statements. The Company cannot guarantee the accuracy of the forward-looking statements, and you should be aware that the Company's actual results could differ materially from those contained in the forward-looking statements due to a number of factors, including the statements under "Risk Factors" contained in the Company's reports filed with the Securities and Exchange Commission.

#### **Item 9.01 Financial Statements and Exhibits**

(d) Exhibits

Exhibit No. Description

99.1 Slide presentation of NeoStem, Inc. dated September 2014\*

\*Exhibit 99.1 is furnished as part of this Current Report on Form 8-K.

#### SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

#### NEOSTEM, INC.

By: /s/ Catherine M. Vaczy

Name: Catherine M. Vaczy, Esq.

Title: General Counsel

Dated: September 8, 2014



#### FORWARD-LOOKING STATEMENTS



This presentation contains "forward-looking" statements within the meaning of the Private Securities Litigation Reform Act of 1995, as well as historical information. Such forward-looking statements involve known and unknown risks, uncertainties and other factors which may cause our actual results, performance or achievements, or industry results, to be materially different from anticipated results, performance or achievements expressed or implied by such forward-looking statements. When used in this presentation, statements that are not statements of current or historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, the words "plan," "intend," "may," "will," "expect," "believe," "could," "anticipate," "estimate," or "continue" or similar expressions or other variations or comparable terminology are intended to identify such forward-looking statements, although some forward-looking statements are expressed differently. We remind readers that forward-looking statements are merely predictions and therefore inherently subject to uncertainties and other factors and involve known and unknown risks that could cause the actual results, performance, levels of activity or our achievements or industry results, to be materially different from any future results, performance levels of activity or our achievements or industry results expressed or implied by such forward-looking statements appear in this presentation. Factors that could cause our actual results of differ materially from anticipated results expressed or implied by forward-looking statements include, among others:

- our ability to obtain sufficient capital or strategic business arrangements to fund our operations and expansion plans, including meeting our financial obligations under various licensing and other strategic arrangements, the funding of our clinical trials for product candidates in our development programs for our Targeted Caner Immunotherapy Program, our Ischemic Repair Program and our Immune Modulation Program, and the commercialization of the relevant technology;
- · our ability to build and maintain the management and human resources infrastructure necessary to support the growth of our business;
- · our ability to integrate our acquired businesses successfully and grow such acquired businesses as anticipated, including expanding our PCT business internationally;
- · whether a large global market is established for our cellular-based products and services and our ability to capture a meaningful share of this market;
- · scientific and medical developments beyond our control;
- our ability to obtain and maintain, as applicable, appropriate governmental licenses, accreditations or certifications or comply with healthcare laws and regulations or any other adverse effect or limitations caused by government regulation of our business;
- whether any of our current or future patent applications result in issued patents, the scope of those patents and our ability to obtain and maintain other rights to technology required or desirable for the conduct of our business; our ability to commercialize products without infringing the claims of third party patents;
- · whether any potential strategic or financial benefits of various licensing agreements will be realized;
- the results of our development activities, including the results of our planned Intus Phase Phase 3 clinical trial of DC/TC being developed to treat metastatic melanoma, our PreSERVE Phase 2 clinical trial of AMR-001 being developed to treat acute myocardial infarction and planned clinical trials;
- our ability to complete our other planned clinical trials (or initiate other trials) in accordance with our estimated timelines due to delays associated with enrolling patients
  due to the novelty of the treatment, the size of the patient population and the need of patients to meet the inclusion criteria of the trial or otherwise;
- the other factors discussed in "Risk Factors" in our Form 10-K filed with the Securities and Exchange Commission ("the SEC") on March 13, 2014, and elsewhere in the Annual Report on Form 10-K; and
- the Company's acquisition of California Stem Cell, Inc. ("CSC Acquisition") and the ongoing operations associated with this new business will subject the Company to
  additional risks. Our Current Report on Form 8-K filed on May 8, 2014 reporting the closing of the CSC Acquisition contains a discussion of the risk factors related to the
  CSC Acquisition and our new Targeted Immunotherapy Program.

The factors discussed herein, including those risks described in Item 1A. "Risk Factors" in the Company's Annual Report on Form 10-K filed with the SEC on March 13, 2014, the "Risk Factors" described in the Current Report on Form 8-K filed by the Company on May 8, 2014 and in the Company's other periodic filings with the Securities and Exchange Commission (the "SEC") which are available for review at <a href="https://www.sec.gov">www.sec.gov</a> under "Search for Company Filings" could cause actual results and developments to be materially different from those expressed or implied by such statements. All forward-looking statements attributable to us are expressly qualified in their entirety by these and other factors. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. Except as required by law, the Company undertakes no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.



## **CELL THERAPY**

Using cells to prevent or treat disease and modulate the immune system Holds the promise to dramatically transform the course of medicine

Improve clinical outcomes

Reduce overall healthcare costs



## **ABOUT NEOSTEM**

Leader in the emerging cellular therapy industry developing novel proprietary cell therapy products as well as generating revenue through a contract development and manufacturing organization that we believe will benefit from the growth of this industry

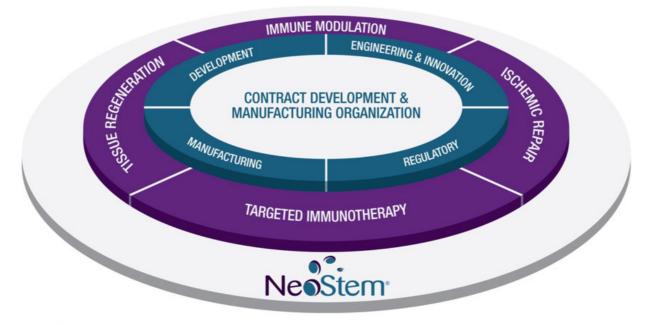
- Integrated entity with platform technologies, a strong pipeline, and a revenue-generating contract development and manufacturing service business
- Recently expanded pipeline into cancer immunotherapies through acquisition of California Stem Cell, Inc. with Phase 3 candidate
- Over \$33M in cash as of June 30, 2014
- Headquarters in New York City
- GMP-compliant facilities in Allendale, NJ; Mountain View, CA; and Irvine, CA
- 156 employees as of August 6, 2014



## NEOSTEM HAS AN INTEGRATED BUSINESS MODEL



Therapeutic development around a significant IP portfolio and a revenue-generating service business allows for cost effective inhouse product development and immediate revenue





### DEVELOPMENT HIGHLIGHTS: MULTIPLE PLATFORM TECHNOLOGIES



DEVELOPING A PORTFOLIO OF CELL THERAPY PRODUCTS THAT LEVERAGES THE BODY'S NATURAL ABILITY TO HEAL AND FIGHT DISEASE

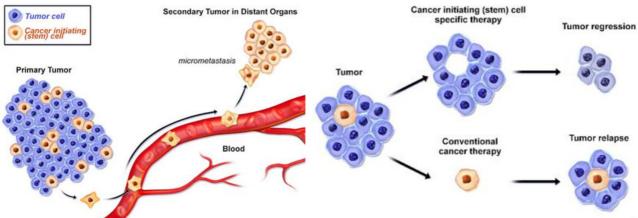
- TARGETED CANCER IMMUNOTHERAPY PROGRAM
  - Using DC/TC Technology
- ISCHEMIC REPAIR PROGRAM
  - Using CD34 Cell Technology
- IMMUNE MODULATION PROGRAM
  - Using T Regulatory Cell Technology
- TISSUE REGENERATION PROGRAM
  - Using VSEL<sup>™</sup> Technology and Stem Cell Derived Growth Factors



## TARGETED CANCER IMMUNOTHERAPY PROGRAM RATIONALE



Cancer initiating (stem) cells\* can move through the blood stream to form new metastasis and grow to form new tumors



- Cancer initiating (stem) cells isolated from patient tumor provide potent signature antigens to educate and direct the immune system
- Immunotherapy product uniquely targets the patient's cancer initiating (stem) cells,
   which are otherwise capable of reconstituting the tumor
- Therapies that fail to target cancer initiating (stem) cells are not likely to prevent recurrence of tumors

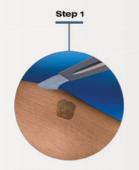


\* These cells are defined as invasive migratory cancer initiating cells capable of reconstituting and developing new tumors

## TARGETED CANCER IMMUNOTHERAPY TREATMENT PROCESS



Step 6



#### STEP 1: Creation of the treatment begins with the surgical resection of the patient's tumor



Step 2

STEP 2: (DAY 0 - WEEK 6)

The cancer initiating (stem) cells from the tumor are isolated, expanded, and irradiated to render them inactive



STEP 3: (PRIOR TO WEEK 6) Patient undergoes leukapheresis, a standard procedure in which monocytes are extracted from circulating blood



Step 4

STEP 4: (WEEK 6)
Monocytes mature
into dendritic cells,
and are exposed to
the irradiated cancer
initiating (stem) cells,
learning how to
identify cancer
initiating (stem) cells
based on their
antigen signature



STEP 5: (WEEK 6 - WEEK 8) Mature, reactive dendritic cells are cryopreserved, quality controlled, then shipped to the clinical site



STEP 6: When convenient for the clinician, treatment begins (includes eight injections administered over the course of six months)

## FEATURES OF OUR TARGETED CANCER IMMUNOTHERAPY PROGRAM



#### **OUR IMMUNOTHERAPY**

Presents the entire spectrum of patient-specific cancer initiating (stem) cells for the immune system to target

Targets the cancer initiating (stem) cells that express antigens associated with mutated cell lineages

Induces or enhances persistent T-cell immunity with activated dendritic cells

Uses autologous cancer antigen immune priming

#### **OVERCOMES**

Limited antigen targeting

Tumor mutation/escape

Weak immune response

Toxicity



### FIRST TARGET INDICATION: MELANOMA



#### **BASICS OF MELANOMA**

- Most lethal form of skin cancer
- Most often caused by unrepaired DNA damage to skin cells from UV radiation
- 120,000 new cases per year in U.S.<sup>1</sup>
- Kills an estimated 8,790 in U.S. annually<sup>2</sup>

#### **SURVIVAL RATE**

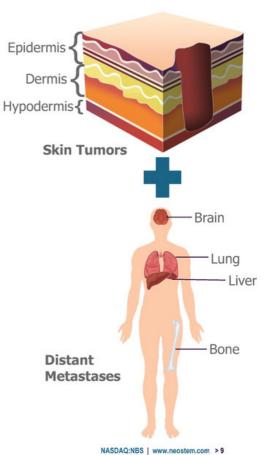
 Stage IV metastatic melanoma – 15% five-year survival rate with current therapies<sup>3</sup>

## CURRENT MAJOR-MARKET\* LANDSCAPE FOR MALIGNANT MELANOMA

- 111,520 newly diagnosed patients
- 2012 Total: \$950 million
- 76% of cost is spent on immunotherapies

American Cancer Society
 Skin Cancer Foundation
 ANCC Cancer Staging 2010 (based on 17 academic centers)
 All other data from Decision Resources Malignant Melanoma – 2013 Report
 \* U.S., Europe and Japan





## **OTHER THERAPEUTICS** FOR MELANOMA



THERAPY	2 YR OVERALL SURVIVAL	SIDE EFFECTS	ESTIMATED COST
Proleukin (Interleukin-2) Prometheus Labs	25%1	Capillary Leak Syndrome Impaired Neutrophil Function Disseminated Infection Sepsis	>\$100,000
Yervoy (Ipilimumab) (CTLA-4 inhibitor) Bristol Myers – Squibb	28%²	Enterocolitis Hepatitis Dermatitis Neuropathy Endocrinopathy GI Disorders	>\$100,000
Oral BRAF inhibitors & MEK inhibitors	28%³	Cutaneous Malignancies Hypersensitivity Reactions Tumor Promotion in BRAF wild-type QT Prolongation Hepatotoxicity	>\$100,000
Chemotherapy	15%4	Anemia Fatigue Risk of Infection Nausea/Diarrhea/Constipation	~\$50,000
1 Fton 700 200	12 Atking 7/22 2009		

<sup>1.</sup> Eton JCO 2002, Atkins JCO 2008

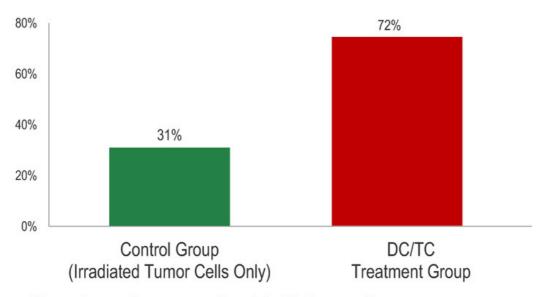
<sup>2.</sup> Hodi NEJM 2010, Robert NEJM 2010, Wolchok Ann Oncol 2013

NeoStem  $4. \ Chapman \ \textit{JCO}\ 1999, \ Middleton \ \textit{JCO}\ 2000, \ Ranson \ \textit{JCO}\ 2007, \ Robert \ \textit{NEJM}\ 2011, \ Chapman \ \textit{NEJM}\ 2011 \ (Derived from a range of 9-20\%)$ 

# PHASE 2 RESULTS FOR DC/TC PRODUCT CANDIDATE FOR METASTATIC MELANOMA



#### **2 YEAR OVERALL SURVIVAL**



- No serious adverse events related to immunotherapy
- · Minor local injection site reactions

Dillman, et al. Journal Immunotherapy 2012



## INTUS PHASE 3 SPECIAL PROTOCOL ASSESSMENT (SPA) STUDY DESIGN



STUDY NAME

Ithe TUS study

TARGET Patients with Stage IV or recurrent Stage III metastatic melanoma

LOCATION United States and potentially Australia & New Zealand,

approximately 60 sites

Design Double blind, placebo controlled, randomized (2:1), intent to treat

analysis, planned enrollment 250 evaluable patients; 80% power to

detect 37.5% reduction in risk of death; Hazard ratio=0.625

**ENDPOINT** Overall survival

TREATMENT GROUP DC/TC (autologous dendritic cells pulsed with irradiated tumor cells

in GM-CSF)

CONTROL GROUP Autologous mononuclear cells (MC) in GM-CSF

SPECIAL PROTOCOL Indicates FDA is in agreement with the design, clinical endpoints and planned clinical analysis of this Phase 3 trial and could serve

as the basis for a Biologics License Application



### ISCHEMIC REPAIR PROGRAM: ENHANCING THE BODY'S NATURAL REPAIR MECHANISM



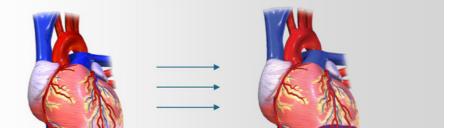
- Ischemia occurs when supply of oxygenated blood in body is restricted
- We seek to reverse this restriction through development and formation of new blood vessels
- CD34/CXCR4 cells are a natural repair mechanism, following the SDF gradient towards the area of need
- This natural repair mechanism works the same for multiple areas of vascular insufficiency such as:
  - Acute myocardial infarction
  - Traumatic brain injury
  - Chronic heart failure
  - Critical limb ischemia



## NBS10 (AMR-001) FOR POST-STEMI TREATMENT



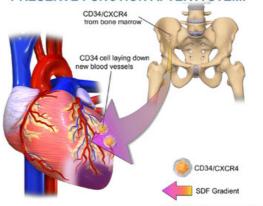
- Following a heart attack, apoptosis and progressive cardiomyocyte loss leads to infarct expansion
- ST segment Elevation MI (STEMI) patients are at a high risk of a progressive deterioration in heart muscle function that leads to worsening of heart function, morbidity and mortality



THE NATURAL PROGRESSION OF DISEASE POST-STEMI

#### NBS10 BRINGS REPAIR SYSTEM TO THE HEART TO PRESERVE FUNCTION AFTER A STEMI

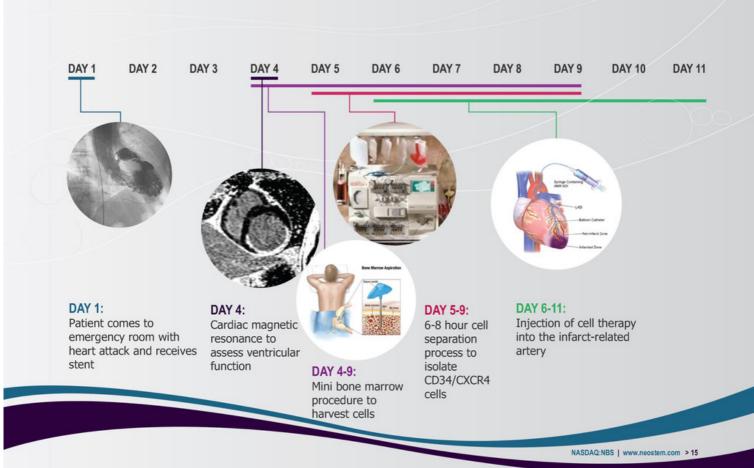
Peri-Infarct Zone





## PRESERVE PHASE 2 STUDY TREATMENT PROCESS





## PHASE 1 RESULTS POINT TO NBS10 POTENTIAL



## DOSE RESPONSE CORRELATED WITH MOBILE CD34 CELLS

Patients dosed ≥ the threshold dose of 10 million cells showed significant improvement in perfusion

#### RTSS (HYPOPERFUSION)

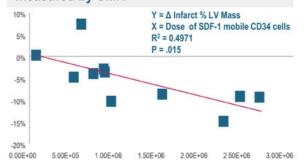
COHORT	BASE LINE	6 MONTHS	DELTA	% CHANGE
Control	259.0	273.5	+14.5	+5.6
5M Cells	714.2	722.0	+7.8	+1.1
10M Cells	998.6	635.8	-362.8	-36.4
15M Cells	584.0	462.0	-122.0	-20.9

## DSMB DETERMINED THAT THERE WERE NO SAFETY CONCERNS THAT WARRANTED ANY ACTION

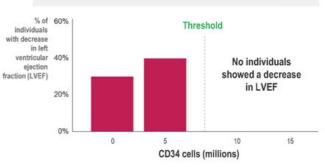
Quyyumi AmHtJ 2011 and data on file



## Increasing doses of CD34/SDF-1 mobile cells reduced the size of the infarct region as measured by CMR



## At threshold dose of 10 million cells or more, no individuals showed decrease in LVEF



### PRESERVE PHASE 2 STUDY: ENROLLMENT COMPLETED WITH ANTICIPATED DATA RELEASE 2014



TARGET Post-AMI patients

KEY INCLUSION CRITERIA Confirmation of ST Elevation MI (STEMI); ejection fraction

≤ 48% at day 4; state of the art care post stenting

LOCATION AND NUMBER OF SUBJECTS

United States, 60 centers, 160 patients (enrollment

completed)

**DESIGN** Double blind, placebo controlled, randomized (1:1)

PRIMARY ENDPOINT Change in cardiac perfusion (RTSS by SPECT) from

baseline to 6 months

OTHER ENDPOINTS Secondary endpoints to determine preservation of cardiac

function and clinical events:

 CMR to measure LVEF, LVESV, LVEDV, regional myocardial strain, infarct/peri-infarct regional wall motion abnormalities, and infarct size (baseline and 6

months)

Quality of Life measures: (KCCQ & SAQ)

■ Reduction in cumulative MACE and other adverse clinical cardiac events at 6, 12, 18, 24, and 36 months

TREATMENT Single dose via infarct related artery with minimum dose

for release >10MM CD34+ cells

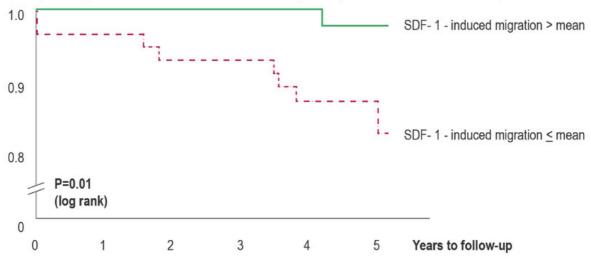
NeoStem

# MIGRATORY CAPACITY OF ADMINISTERED CD34 CELLS ASSOCIATED WITH EVENT-FREE SURVIVAL POST AMI



 Recently published study demonstrated administration of autologous SDF-1 migratory CD34 cells, significantly reduces cumulative incidence of major adverse clinical cardiac events following acute myocardial infarction (AMI)

Event-free survival (%) (cardiac, cardiovascular and unknown death, rehospitalization for heart failure)



Assmus, B., et al. (2014) Long-term clinical outcome after intracoronary application of bone marrow-derived mononuclear cells for acute myocardial infarction: migratory capacity of administered cells determines event-free survival. European Heart Journal



## FEATURES AND BENEFITS OF NBS10



#### **FEATURES**

- CD34/CXCR4 cells home to the viable tissue surrounding the infarcted (dead) myocardium (peri-infarct zone) after administration and persist
- Autologous cells take up residence in the peri-infarct zone, likely promoting angiogenesis (development and formation of new blood vessels)
- Cell preparation has a 72 hour shelf life and is infused into patient 5 to 11 days following an acute myocardial infarction (AMI)
  - ▶ After the pro-inflammatory "hot phase"
  - ▶ Prior to permanent scar formation

#### **BENEFITS**

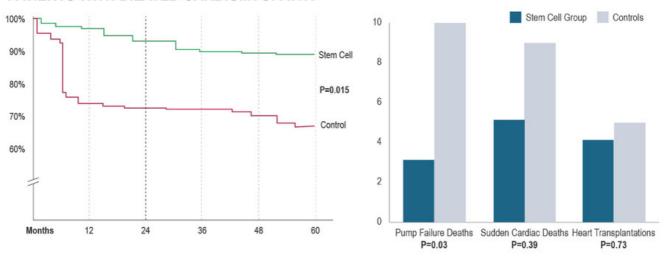
- Amplifies the body's natural repair mechanism
- Cells are not expanded no risk of genetic mutation
- Cells are autologous no immunogenicity risk
- Delivery where cells are needed without having to inject into myocardium
  - ▶ Safer and greater distribution



### RECENT DATA SUPPORTS CD34 STEM CELL THERAPY IN CHRONIC HEART FAILURE



## CD34 STEM CELL THERAPY SIGNIFICANTLY IMPROVES EVENT-FREE SURVIVIAL AT 5 YEARS IN PATIENTS WITH DILATED CARDIOMYOPATHY



- Significant need prevalence of over 23 million worldwide, 5.7 million U.S.
- Therapy would enable larger distribution (not limited to mapping systems)

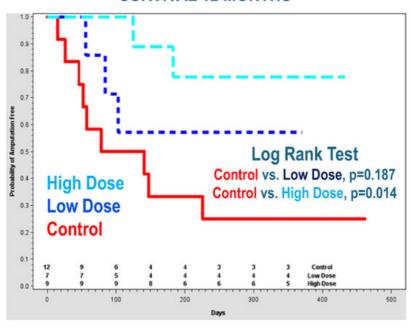
Adapted from Vrtovec et al, *Circ Res* published online 10/12/2012 Note: 110 patients (open label, 55 treated with cells and 55 standard of care)



# RECENT DATA SUPPORTS CD34 STEM CELL THERAPY IN CRITICAL LIMB ISCHEMIA



## PROBABILITY OF AMPUTATION-FREE SURVIVAL 12 MONTHS



- Double blind, randomized, controlled trial of autologous CD34 cells
- Two dose levels (N=28); Diabetics distributed equally
- CLI Patients (Rutherford Score IV or V); Non-optimal candidate for surgical or percutaneous revascularization or have refused revascularization
- 8 intramuscular injections or placebo Rx

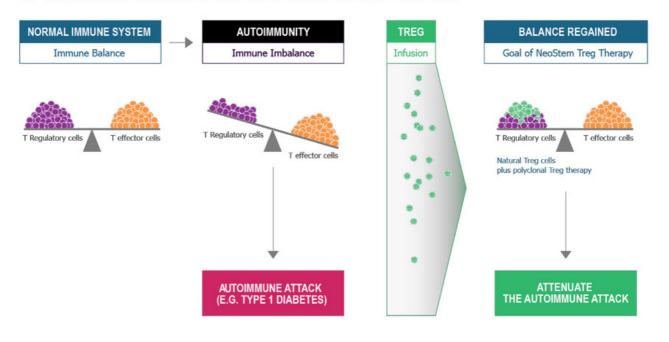
Losordo et al. (2012) A Randomized, Controlled Pilot Study of Autologous CD34+ Cell Therapy for Critical Limb Ischemia, Circulation Cardiovascular Interventions.



## IMMUNE MODULATION PROGRAM: POTENTIAL TO LIMIT AUTOIMMUNITY



## TREG THERAPY REPRESENTS A NOVEL APPROACH FOR RESTORING IMMUNE BALANCE BY ENHANCING T REGULATORY CELL NUMBER AND FUNCTION<sup>1</sup>



1. Chai, Jian-Guo et al, Journal of Immunology 2008; 180;858-869



## FEATURES OF OUR IMMUNE MODULATION PROGRAM



#### **FEATURES OF TREGS:**

- Natural part of immune system
- Regulate activity of T effector cells (responsible for protection from viruses and foreign antigens)
- In autoimmune disease it is thought that deficient Treg activity permits the T effector cells to attack the body's own tissues

#### SIGNIFICANT COLLABORATIONS:

■ Partnership with Becton Dickinson (11.5% program ownership)



 Accelerated development through collaboration with University of California, San Francisco and laboratory of Dr. Jeffrey Bluestone





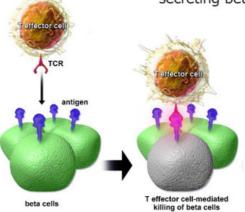
## FIRST TARGET INDICATION: DIABETES MELLITUS TYPE-1 (T1D)



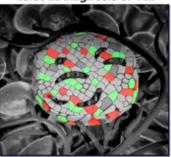
- Also called insulin dependent diabetes or juvenile diabetes
- Affects >34 million worldwide, 1 in 300 children and more adults
- Economic burden of T1D in the U.S. is estimated at \$14.9 billion
- Autoimmune destruction of insulinproducing (beta cells) of the pancreas
- Diabetes is leading cause of kidney failure, new cases of adult blindness, and nontraumatic lower-limb amputations
- Results in total insulin deficiency
- At time of diagnosis, there are still insulinsecreting beta cells in islets







Islet at diagnosis of T1D

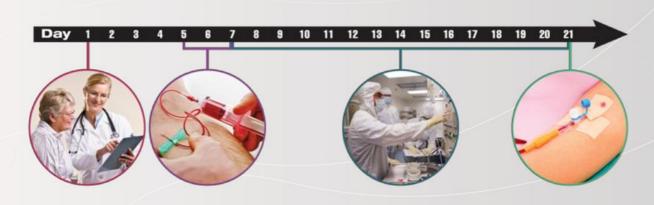


Insulin-secreting cells Glucagon-secreting cells



## **T1D TREG TREATMENT PROCESS**





DAY 1: Screening and enrollment

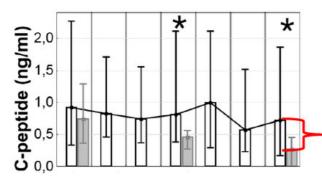
DAY 5-7: Blood draw from patient

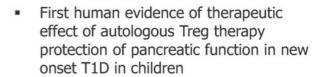
DAY 7-21: Manufacturing including expansion

DAY 21: Infusion of Treg therapy to patient

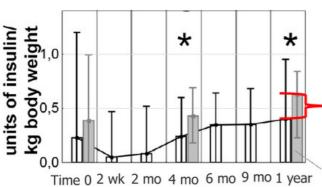
# ADMINISTRATION OF REGULATORY T CELLS PRESERVES BETA CELL FUNCTION IN T1D IN CHILDREN\*







One year follow-up: evidence that Treg therapy preserves function of pancreatic islets cells



Marek-Trzonkowska N et al. *Diabetes Care* 2012;35:1817-1820 Marek-Trzonkowska N et al. *Clinical Immunology* 2014

- C-peptide levels stabilized
- Reduction of insulin requirements

20% of patients able to come off of exogenous insulin four months after treatment



\* Children aged 8-16 in study Regulatory T cells expressing CD4+CD25<sup>high</sup>CD127

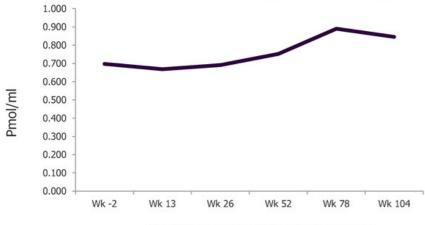


### ADMINISTRATION OF REGULATORY T CELLS\* APPEARS TO BE SAFE IN ADULTS WITH ESTABLISHED T1D



- Preliminary data indicates safety and tolerability
- Infused Tregs detected in peripheral circulation for over 6 months
- Results complement safety and efficacy data from new onset trial in children and informs design of NeoStem's Phase 2 trial in new onset T1D

#### Mean C-peptide levels (MMTT AUC\*\*)



Summary data of 4 dose cohorts (14 patients) through completed follow up through 104 weeks

\* Regulatory T cells expressing CD4+CD25highCD127\*

\*\* MMTT = Mixed Meal Tolerance Test

AUC = Area under the curve

Gitelman et al, American Diabetes Association Abstract, 2014



### **MARKET OPPORTUNITY IN ASTHMA**



### **ASTHMA**

- Affects 25 million in U.S. and 300 million worldwide
- Asthma accounts for \$56 billion in annual direct and indirect health care costs in U.S.
- Steroid resistant asthma afflicts less than 5% of the total asthma population, but accounts for up to 50% of healthcare spending on asthma
- Plan to initiate proof-of-concept study subject to review and approval of the protocol by the appropriate regulatory authorities

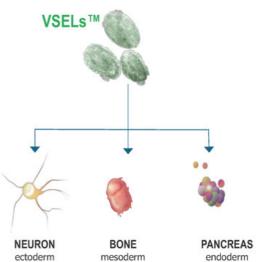


### **TISSUE REGENERATION PROGRAMS**



## VSEL™ TECHNOLOGY: POTENTIAL TO REPAIR DAMAGED TISSUE

- Evaluating therapeutic potential of very small embryoniclike stem cells (VSELs<sup>™</sup>)
- Research suggests multipotency and multi-lineage differentiation into all basic cell types (mesoderm, ectoderm, endoderm)
- Exploring the development for retinal repair and the treatment of chronic wounds
- \$4.5 million of grants toward preclinical VSEL<sup>™</sup> research



## DERMATOLOGY PROGRAM: TOPICAL PRODUCT BASED ON STEM CELL DERIVED GROWTH FACTORS

 Exploring potential for fine lines and wrinkles, psoriasis, and wound care





### INTELLECTUAL PROPERTY



#### TARGETED CANCER IMMUNOTHERAPY PROGRAM

- 1 issued patent and 28 pending patents in the U.S. and OUS with coverage including:
  - ▶ Stem cell growth medium and methods of making and using same; Antigen-presenting cancer vaccines; Individualized high purity carcinoma initiating (stem) cells for target indications, methods and use of same; and rapid methods to produce high purity cancer initiating (stem) cells

#### ISCHEMIC REPAIR PROGRAM

- Broad and growing patent portfolio supports cardiac conditions and a broad range of other conditions caused by underlying ischemia
- 16 granted or allowed composition of matter and methods patents
- Patent Applications: 20 U.S. and OUS patents pending

#### **IMMUNE MODULATION PROGRAM**

- Exclusive rights to 23 issued patents covering isolation, activation, expansion and methods of treating or preventing certain conditions and/or diseases using Tregs in U.S. and major international markets
- Includes composition of matter patents and method patents

#### TISSUE REGENERATION (VSEL™ TECHNOLOGY)

■ In-licensed from the University of Louisville the world-wide patent rights and know-how regarding the isolation, purification and therapeutic use of very small embryonic-like (VSEL<sup>™</sup>) stem cells



## **CLINICAL TIMELINES**



	Jan 1	Apr 1	Jul 1	Oct 1	Dec 31
Targeted Cancer Immunotherapy Program	Q1	Q2	Q3	Q4	
Stage IV or recurrent Stage III melanoma					
- Initiate Phase 3 clinical trial					
Ischemic Repair Program	Q1	Q2	Q3	Q4	
Acute myocardial infarction					
- Phase 2 data <sup>1</sup>					
Immune Modulation Program	Q1	Q2	Q3	Q4	
Type 1 diabetes					
- Initiate Phase 2 clinical trial <sup>2</sup>					
Steroid resistant asthma					
- Initiate Phase 1 clinical trial <sup>2</sup>					1

<sup>1.</sup> An abstract for the PreSERVE AMI study has been accepted for presentation at the American Heart Association's Scientific Sessions being held November 15-19, 2014 although we anticipate results of the study will be released earlier

<sup>2.</sup> Subject to review and approval of the protocols by the appropriate regulatory authorities



# PCT PROVIDES OUTSOURCED MANUFACTURING CAPABILITIES TO CELL THERAPY INDUSTRY



- High quality manufacturing capabilities with 15-year track record of success
- Proven efficiencies and reduced capital investment for customers through outsourcing
- Demonstrated regulatory expertise:
  - ▶ 50+ EU and U.S. regulatory filings;
  - ► All clinical trial phases including BLA submission and product approval by FDA
- Significant focus on innovation, engineering and automation
- EU product distribution requirement compliant
- Continuing to expand commercial capabilities in the U.S. and internationally





ALLENDALE, NEW JERSEY (30,000 ft²)
ISO Class 7 / Class 10,000 suites
ISO Class 6 / Class 1,000 suite
Recent expansion of clean room space

MOUNTAIN VIEW, CALIFORNIA (25,000 ft²) ISO Class 7 / Class 10,000 suites Recent expansion of clean room space

IRVINE, CALIFORNIA (12,500 ft²) ISO Class 7 / Class 10,000 suites



# CONTRACT MANUFACTURING IS A SIGNIFICANT OPPORTUNITY



#### **EXAMPLES OF CONTRACT SERVICES POTENTIAL FROM CONCEPTION TO COMMERCIALIZATION\***

	LOW COMPLEXITY PRODUCT	MEDIUM COMPLEXITY PRODUCT	HIGH COMPLEXITY PRODUCT
PRECLINICAL DRUG DISCOVERY CONTRACT	12 to 18 Month Engagement	12 to 24 Month Engagement	24 to 36 Month Engagement
	\$50,000 to \$250,000	\$250,000 to \$500,000	\$500,000 to \$1,000,000
PHASE 1 CLINICAL TRIAL MANUFACTURING CONTRACT	6 to 12 Month Eng.	12 to 18 Month Eng.	12 to 24 Month Eng.
	5 to 25 Units Produced	25 to 50 Units Produced	50 to 100 Units Produced
	\$250,000 to \$750,000	\$625,000 to \$1,250,000	\$1,000,000 to \$2,000,000
PHASE 2 CLINICAL TRIAL MANUFACTURING CONTRACT	12 to 18 Month Eng.	12 to 24 Month Eng.	18 to 36 Month Eng.
	25 to 50 Units Produced	100 to 200 Units Produced	200 to 400 Units Produced
	\$625,000 to \$1,250,000	\$2,000,000 to \$4,000,000	\$3,000,000 to \$6,000,000
PHASE 3 CLINICAL TRIAL MANUFACTURING CONTRACT	12 to 18 Month Eng.	24 to 48 Month Eng.	24 to 48 Month Eng.
	50 to 100 Units Produced	200 to 400 Units Produced	400 to 1,000 Units Produced
	\$1,000,000 to \$2,000,000	\$3,000,000 to \$6,000,000	\$4,000,000 to \$10,000,000
COMMERCIAL MANUFACTURING CONTRACT	Est. Peak Annual Sales	Est. Peak Annual Sales	Est. Peak Annual Sales
	2,500 to 5,000 Units	10,000 to 25,000 Units	25,000 to 50,000 Units
	\$38M to \$75M / Yr.	\$80M to \$200M / Yr.	\$125 to \$250M / Yr.

 $<sup>{}^{*}\</sup>text{Based}$  on industry experience and estimated potential future commercial manufacturing in the industry



### MANAGEMENT HIGHLIGHTS



#### Robin Smith, MD, MBA - Chief Executive Officer

- Leading NeoStem since 2006, completed six acquisitions and one divestiture; Raised over \$190 million
- Extensive experience in executive and board level capacities for medical enterprises and healthcare-based entities

#### Robert Dickey IV, MBA - Chief Financial Officer

 15+ years management experience at life science companies, including cell therapy experience as CFO of StemCyte, following a career as an investment banker at Lehman Brothers

#### Douglas W. Losordo, MD - Chief Medical Officer

Leader in cell therapy research and renowned cardiologist;
 Obtained over \$35 million in NIH funding during career-long efforts to develop novel therapeutics

#### Andrew L. Pecora, MD - Chief Visionary Officer

 Chief Innovations Officer at John Theurer Cancer Center at Hackensack University Medical Center; Co-founder of PCT; Significant experience in design and conduct of clinical trials

#### Robert A. Preti, PhD - Chief Scientific Officer, President of PCT

 Leading authority on cell engineering; Co-founder of PCT; 10 years prior experience as Director of Hematopoietic Stem Cell Processing & Research Laboratory

#### Hans Keirstead, PhD - President, NeoStem Oncology

 15+ years of experience; CEO of California Stem Cell prior to acquisition; Founder of Stem Cell Research Center, University of California at Irvine; Previously Professor, UCI; Previously CEO of Ability Biomedical

#### Stephen W. Potter, MBA - Executive Vice President

 Biotech and pharma experience: Osiris Therapeutics (approval of Prochymal®, first-ever stem cell drug therapy), Genzyme, DuPont Pharmaceuticals, Booz Allen & Hamilton

#### David Altarac, MD, MPA - VP, Regulatory Affairs

 Extensive experience in U.S. and global regulatory affairs, including strategy, operations, labeling and departmental leadership; 13 year tenure at Merck

#### Robert Dillman, MD - VP, Oncology

 CMO of California Stem Cell prior to acquisition; Executive Medical Director of the Hoag Hospital Institute for Research and Education and Clinical Professor of Medicine at UC Irvine

#### Adel Nada, MD - VP, Immunotherapy

 Formerly Senior Medical Director, Cardiovascular Cell Therapies at Baxter Healthcare; Led Clinical Pharmacology Medical Dept. at Abbott Laboratories

#### Catherine M. Vaczy, Esq. - General Counsel

 Senior business executive and counsel with 20+ years of leadership experience in the biotech industry; Former senior executive at ImClone Systems (\$1 billion co-development deal in oncology forged with Bristol-Myers Squibb)



## **BOARD OF DIRECTORS**



Robin Smith, MD, MBA Chairman of the Board	<ul> <li>MD – Yale; MBA – The Wharton School</li> <li>Formerly President &amp; CEO IP2M, EVP &amp; CMO HealthHelp</li> <li>Experience - Trustee of NYU Medical Center; Chairman of the Board of NYU Hospital for Joint Diseases (through November 2009) and Chairman of Stem for Life Foundation</li> </ul>		
Richard Berman	■ BS and MBA - NYU; JD - Boston College		
Independent Director	<ul> <li>Over 35 years of venture capital, management, M&amp;A experience</li> </ul>		
	<ul> <li>Experience – Current Board of Directors of Apricus Biosciences, Easylink Services International, Inc., Advaxis, Inc., Broadcaster, Inc., National Investment Managers</li> </ul>		
Drew Bernstein, CPA	BS – University of Maryland Business School		
Independent Director	<ul> <li>Licensed in State of New York; member AICPA, NYSSCPA and NSA</li> </ul>		
	<ul> <li>Experience – Bernstein &amp; Pinchuk LLP (member of BDO Seidman Alliance); PRC auditing; 200+ real estate transactions with \$3B+ aggregate value; accountant and business advisor</li> </ul>		
Martyn Greenacre, MBA	BA – Harvard College; MBA – Harvard Business School		
Independent Director	<ul> <li>Experience – Board and executive positions for multiple biopharmaceutical companies; Former CEO of Delsys Pharmaceutical Corporation and Zynaxis Inc; Chairman of the Board of BMP Sunstone Corporation</li> </ul>		
Steven M. Klosk	■ BS Industrial & Labor Relations – Cornell; JD – New York Law School		
Independent Director	<ul> <li>Experience – President, CEO &amp; Director of Cambrex Corporation (leading provider of active pharmaceutical ingredients) since 2008 driving significant revenue growth during his tenure</li> </ul>		
Steven Myers	BS Mathematics – Stanford University		
Independent Lead Director	<ul> <li>Experience – Founder/Chairman/CEO SM&amp;A (competition management services); career in aerospace and defense sectors supporting DoD &amp; NASA programs</li> </ul>		
Andrew Pecora, MD, FACP	<ul> <li>MD — University of Medicine and Dentistry of New Jersey</li> </ul>		
Director	<ul> <li>Experience – Chief Innovations Officer, Professor and Vice President of Cancer Services at John Theurer Cancer Center at Hackensack University Medical Center, and Managing Partner of the Northern New Jersey Cancer Center</li> </ul>		
Eric Wei	■ BS – Mathematics & Economics – Amherst College; MBA – The Wharton School		
Director	<ul> <li>Experience – Founder/Managing Partner of RimAsia Capital partners (private equity); Formerly with Peregrine Capital, Prudential Securities, Lazard Freres, Citibank, Gilbert Global Equity Partners, and Crimson Asia Capital Partners</li> </ul>		



## **KEY METRICS**



#### **MARKET METRICS**

MARKET CAPITALIZATION <sup>1</sup>	\$203M	
STOCK PRICE <sup>2</sup>	\$5.75	
52 WEEK RANGE <sup>2</sup>	\$4.56 - \$9.89	
FLOAT <sup>1</sup>	31.1M	
INSIDER HOLDINGS <sup>1</sup>	12%	

#### **FINANCIAL METRICS**

REVENUE <sup>3</sup>	\$4.5M (Second Quarter)

CASH<sup>4</sup> \$33.8M

**COMMON SHARES** OUTSTANDING1

35.3M

WARRANTS1 3.6M

(avg. warrant exercise price of \$14.13)

OPTIONS<sup>1</sup> 4.4M

(avg. option exercise price of \$9.27)

<sup>3.</sup> For the three months ended June 30, 2014 4. As of June 30, 2014 (includes marketable securities)



<sup>1.</sup> As of September 1, 2014 (market capitalization based on a \$5.75 share price)

<sup>2.</sup> As of September 1, 2014

## **UNIQUE BUSINESS MODEL**



## COMBINATION OF A LATE STAGE CLINICAL PIPELINE AND A REVENUE-GENERATING SERVICE BUSINESS

#### CANCER TREATMENT - TARGETED IMMUNOTHERAPY PROGRAM

■ Stage IV and recurrent Stage III melanoma – Intus Phase 3 study approved, initiating 2H 2014

#### ISCHEMIC REPAIR - CD34 CELL PROGRAM

■ Acute myocardial infarction — PreSERVE Phase 2 study (data available 2H 2014)

#### IMMUNE MODULATION - T REGULATORY CELL PROGRAM

- Type 1 diabetes Preparing for Phase 2 study, Phase 1 data readout presented at ADA June 2014
- Steroid resistant asthma Preparing for Phase 1 study in Canada

#### **TISSUE REGENERATION**

- VSEL<sup>™</sup> Technology Macular degeneration, wound healing, bone regeneration preclinical
- Human stem cell derived growth factors for dermatologic applications Skin health, psoriasis, wound care

#### CELL THERAPY MANUFACTURING - PROGENITOR CELL THERAPY

- Cost effective in-house product development and immediate revenue and cash flow generation
- Manufacturing, regulatory, and commercialization expertise for therapeutics development
- Cell therapy automation to lower cost and improve efficiency
- Manufacturing expansion in U.S. and internationally



## **CONTACT INFORMATION**



### NEOSTEM, INC.

NASDAQ: NBS

WWW.NEOSTEM.COM

## ROBIN SMITH, MD, MBA

CHAIRMAN & CEO

PHONE: (212) 584-4174

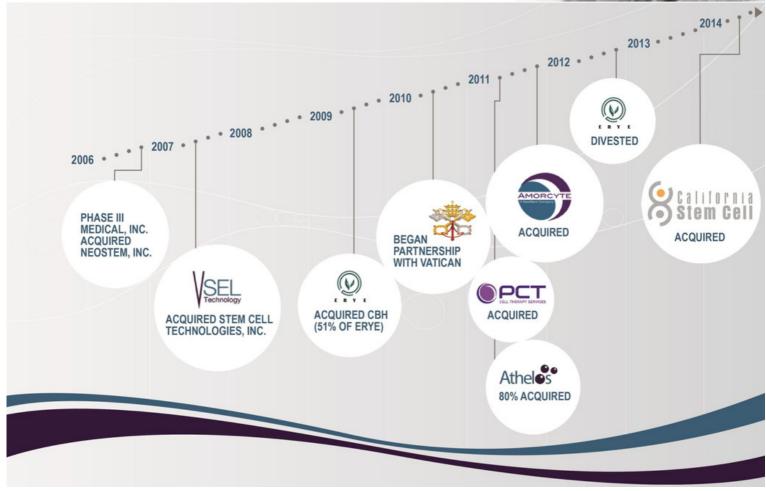
EMAIL: RSMITH@NEOSTEM.COM





## SINCE 2006, ACCESSED OVER \$193M AND COMPLETED MULTIPLE M&A TRANSACTIONS AND ONE DIVESTITURE





## CARDIOVASCULAR SCIENTIFIC ADVISORY BOARD



Chief Medical Officer, NeoStem		
Brigham & Women's Hospital		
The Mayo Clinic		
The Christ Hospital Heart of Greater Cincinnati		
Washington University School of Medicine		
Texas Heart Institute		
University of Michigan School of Medicine		
Emory University School of Medicine		
Emory University School of Medicine		
Texas Heart Institute		
Stanford University School of Medicine		



# IMMUNE MODULATION PROGRAM ADVISORS



The Company accesses these experts to advise in the areas of diabetes, asthma, and other autoimmune conditions for its Immune Modulation Program.

Jeffrey Bluestone, PhD University of California, San Francisco, Diabetes Center

William Busse, MD University of Wisconsin

Mario Castro, MD, MPH Washington University in St. Louis

David A. Horwitz, MD University of Southern California

Robert Korngold, PhD Hackensack University Medical Center

Robert J. Meyer, MD Virginia Center for Translational and Regulatory Sciences

Robert S. Negrin, MD Stanford University

Paul O'Byrne, MB McMaster University

David Peritt, PhD Hospira

Noel L. Warner, PhD BD Biosciences

Prescott Woodruff, MD, MPH University of California, San Francisco

Ne Stem

## VSEL™ TECHNOLOGY ACADEMIC COLLABORATORS



Mariusz Ratajczak, MD, PhD, Dsci University of Louisville

Russell Taichman, DMD, DMSc University of Michigan

Vincent Falanga, MD Boston University

Michael Young, PhD Schepens Eye Research Institute, Harvard Medical School

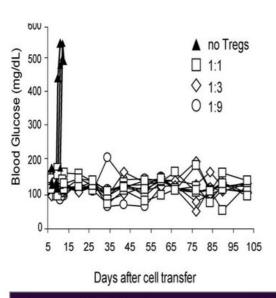
Kameran Lashkari, MD Schepens Eye Research Institute, Harvard Medical School

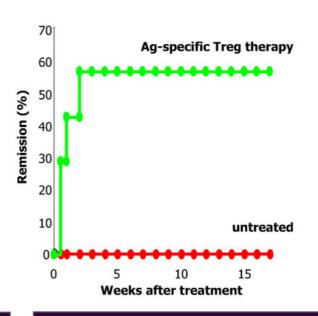
Song Li, PhD University of California, Berkeley



# TREG IMMUNOTHERAPY WORKS IN MODEL OF T1D







Tregs effectively suppress diabetes

**Ag-specific Tregs reverse diabetes** 

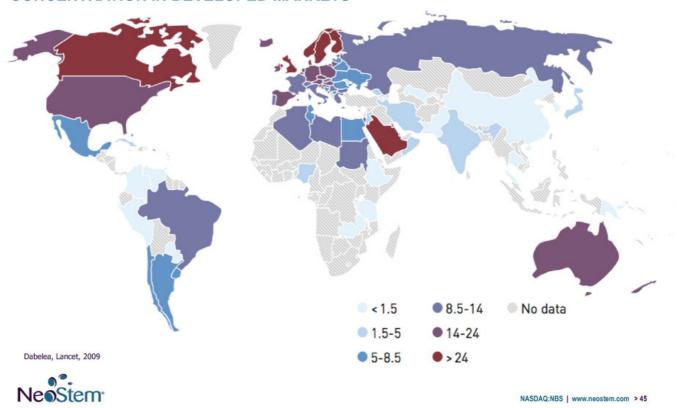
Tang, Bluestone, et al.



## T1D IS ON THE RISE



## NEW CASES OF T1D (0-14 YEARS) PER 100,000 CHILDREN, 2013: CONCENTRATION IN DEVELOPED MARKETS



### **ECONOMIC IMPACT OF T1D**



#### THE ECONOMIC BURDEN OF T1D IN THE U.S. IS ESTIMATED AT \$14.9 BILLION1

Average economic burden per person with diabetes is larger for T1D vs T2D

## PREVENTION IS KEY - MEDICAL COSTS ASSOCIATED WITH T1D INCREASE SUBSTANTIALLY WITH AGE AND DURATION OF DISEASE

- Annual medical costs per person increase with age at a much faster rate for those with T1D vs
   T2D
- For T1D the average medical cost per case increases from ~\$4,000 for people younger than age 44 to ~\$35,000 for the population age 65 and older
- Increased utilization of institutional care in elderly T1D patients

#### \$2 BILLION ESTIMATED MARKET SIZE FOR INSULIN SALES IN 2017

For the T1D indication alone

#### UNMET NEED FOR β-CELL PRESERVING/PREVENTATIVE TREATMENTS FOR T1D

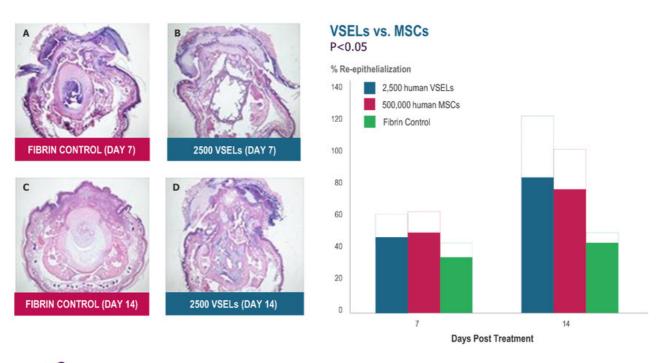
1. Dall TM et al. Population Health Management 2009;12:103-110



## HUMAN VSELS™ ACCELERATE HEALING IN A SCID MOUSE COMPLEX TAIL WOUND MODEL



PRELIMINARY DATA IN A PRECLINICAL MODEL OF SEVERE COMPLEX WOUNDS SUGGEST THAT VSELS™ MAY BE MORE EFFECTIVE IN ACCELERATING HEALING THAN MESENCHYMAL STROMAL CELLS (MSCs)

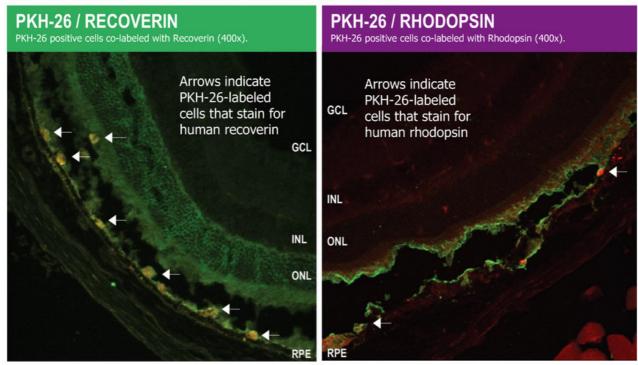




# VSELS™ COULD BE USED TO TREAT MACULAR DEGENERATION



PRELIMINARY DATA SUGGEST HUMAN VSELS™ INJECTED INTO A MOUSE SUB-RETINAL SPACE INTEGRATE AND SHOW DIFFERENTIATION POTENTIAL IN SITU



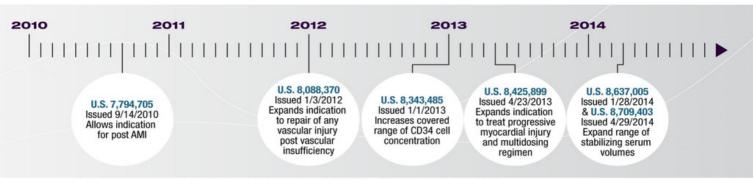
Eminli, S. et al. Exploring the use of human very small embryonic-like stem cells (VSELs) isolated from adult peripheral blood for therapy of dry age-related macular degeneration (AMD). ISSCR 2012 Annual Meeting, Yokohama, Japan. Poster presentation.



# CD34 CELL PROGRAM INTELLECTUAL PROPERTY



- Broad and growing patent portfolio supports cardiac and other ischemic conditions
- NeoStem's patent claims cover a pharmaceutical composition that contains a therapeutic concentration of non-expanded CD34/CXCR4 stem cells that move in response to SDF-1 or VEGF, together with a stabilizing amount of serum, and that can be delivered parenterally through a catheter to repair an injury caused by vascular insufficiency
- Six granted U.S. composition of matter and methods patents



- 10 granted or allowed OUS composition of matter and method patents:
  - ▶ European Union, Japan, South Africa, Malaysia, Philippines, Canada, Russia
- Patent Applications: 20 U.S. and OUS patents pending
- Issued and pending claims can be applied to broad range of other conditions caused by underlying ischemia, including: chronic myocardial ischemia post-AMI; chronic heart failure; critical limb ischemia; and ischemic brain injury

