

## Safe Harbor

This presentation contains forward-looking statements, including, without limitation, statements concerning anticipated progress, objectives and expectations regarding profitability, growth in revenue and earnings per share, cash payments, the commercial potential of our products, intended product development, clinical trial and regulatory plans and progress, objectives and expectations, all of which involve certain risks and uncertainties. These statements are often, but are not always, made through the use of words or phrases such as "anticipates," "intends," "estimates," "plans," "expects," "we believe," "we intend," and similar words or phrases, or future or conditional verbs such as "would," "should," "potential," "could," "may," or similar expressions. Actual results may differ significantly from the expectations contained in the forward-looking statements.

Among the factors that may result in differences are the inherent risks and uncertainties associated with competitive developments, clinical trial and product development activities, regulatory approval requirements, the availability and allocation of resources among different potential uses, estimating the commercial potential of our products and product candidates and growth in revenues and improvement in costs, market demand for our products and our ability to supply or meet customer demand for our products. These and other significant factors are discussed in greater detail in Vericel's Annual Report on Form 10-K for the year ended December 31, 2015, filed with the Securities and Exchange Commission ("SEC") on March 14, 2016, Quarterly Reports on Form 10-Q and other documents filed by the Company with the SEC from time to time.

These forward-looking statements reflect management's current views and Vericel does not undertake to update any of these forward-looking statements to reflect a change in its views or events or circumstances that occur after the date of this release except as required by law.



# Vericel Investment Highlights

Vericel Investment Highlights				
Robust Specialty Biologics Business	<ul> <li>Fully integrated specialty biologics business with strong revenue growth and expanding gross margins</li> <li>Total Carticel® and Epicel® trailing 12-month revenues of \$53.2 million as of Q3 2016         <ul> <li>10% CAGR in revenue since acquisition</li> </ul> </li> </ul>			
Near- and Long-Term Growth Drivers	<ul> <li>MACI® BLA approved by the FDA on December 13, 2016 – potential to significantly expand cartilage repair franchise</li> <li>Epicel HDE supplement approved in February 2016 – revised label includes pediatric patients and probable survival benefit; allows Epicel to be sold for profit</li> <li>Ixmyelocel-T Phase 2b ixCELL-DCM trial for treatment of advanced heart failure due to ischemic DCM met primary endpoint – Fast Track designation granted February 16, 2017</li> </ul>			
Strong Shareholder Base	<ul> <li>Closed \$20 million financing in December 2016</li> <li>Participation by leading institutional healthcare investors</li> </ul>			
Experienced Management Team	<ul> <li>Strong track record of developing and commercializing products in the U.S.</li> <li>Deep experience in restructuring and integrating acquired businesses</li> </ul>			



## Robust Product Portfolio





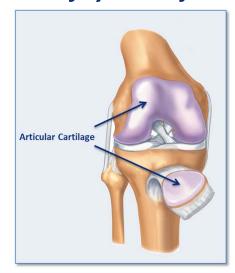
## Overview – Articular Cartilage Structure and Function

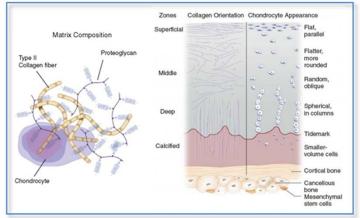
## Articular cartilage is a highly specialized connective tissue of synovial joints

- Articular cartilage function
  - Provides a smooth lubricated surface allowing nearly frictionless movement
  - Facilitates transmission of loads to underlying subchondral bone
  - Protect joints from compressive, tensile and shearing forces



 Chondrocytes are the resident cells responsible for the production, maintenance and repair of ECM

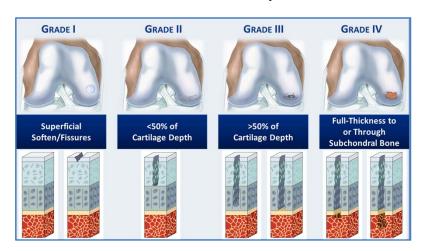






## Articular Cartilage Defects and Treatment Goals

- Articular cartilage injury is a cause of significant musculoskeletal morbidity
  - Cartilage defects are found in ~60% of knee arthroscopies
  - Damage is caused by acute and repetitive trauma, degenerative conditions (OA) and inflammatory conditions (RA)
  - Limited capacity for intrinsic healing and repair
    - Devoid of blood vessels, nerves, or lymphatics
    - Mature chondrocytes have limited potential for replication
  - Untreated lesions may lead to debilitating joint pain, dysfunction, and osteoarthritis



• Treatment Goals: Reduce symptoms, improve function, prevent degeneration

Palliative	Reparative	Restorative		
Techniques intended to relieve or prevent pain with little repair of underlying defect	Marrow-stimulation techniques that result in formation of fibrocartilage	Techniques designed to recreate hyaline-like cartilage at the site of the defect		
Lavage and debridement     Thermal chondroplasty	Microfracture/microdrilling     Augmented microfracture	Autologous chondrocyte implant     Autograft or allograft		



## **MACI** Overview

# MACI is a 3rd generation autologous chondrocyte implant (ACI) for the treatment of cartilage defects of the knee

- First tissue-engineered autologous cellularized scaffold product approved by the FDA (December 2016)
- First tissue-engineered product approved as an Advanced Therapy Medicinal Product by the European Commission (June 2013)<sup>1</sup>



 $^{\rm 1}$  Marketing in the EU has been temporarily suspended.





## MACI Production and Administration

## **MACI Production**



**Biopsy Harvest** 



**Chondrocyte Extraction** 



**Chondrocyte Expansion** 

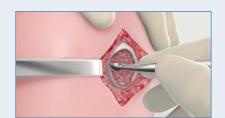


Uniform Cell Seeding

### **MACI Delivered**



## **MACI Administration**



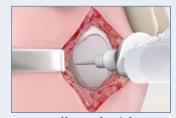
Defect Prepared



Template Created



MACI Implanted



Adhered with Fibrin Glue





# Highlights of MACI Product Attributes vs. Carticel<sup>1</sup>

	Attribute	MACI	Carticel
Label	Indicated Use <sup>2</sup>	First-line treatment	Second-line treatment
Labei	Defect Location <sup>2</sup>	Cartilage defects of the knee	Femoral condyle only
	Implantation procedure	Can administer via mini- arthrotomy	Arthrotomy
Administration	Technical demands of implant procedure	Direct implantation of seeded cellular membrane	Suturing and injection
Clinical Data	Proven clinical efficacy and safety	Statistically significantly greater improvement compared to microfracture	No active control
Rehab Protocol	Rehabilitation	MACI Protocol	Standard ACI Protocol

 $<sup>^1</sup>$  Saris et al., 2014; Vericel, 2015; Welsch et al., 2008; Bachmann et al., 2004; Marlovits et al., 2005.  $^2$  MACI PI; Carticel PI.





# MACI Label Indications and Usage

## 1. Indications and Usage

MACI® (autologous cultured chondrocytes on porcine collagen membrane) is an autologous cellularized scaffold product indicated for the repair of single or multiple symptomatic, full-thickness cartilage defects of the knee with or without bone involvement in adults.

#### Limitations of Use

- Effectiveness of MACI in joints other than the knee has not been established.
- Safety and effectiveness of MACI in patients over the age of 55 years have not been established.

	MACI Label
Indicated Use	First-line treatment
Defect Location	Cartilage defects of the knee, including patella
Defect Size	No limitation
Number of Defects	Single or multiple
Bone Involvement	With or without bone involvement





# **MACI** Administration Advantages

### **CARTICEL**



### Effective in a challenging patient population

 Moderate to large sized chronic, symptomatic lesions that have failed a primary treatment

#### Limitations:

- Technically exacting procedure requiring arthrotomy, periosteal patch harvest and sutures
- Extended surgical time

#### **MACI**



## 3<sup>rd</sup> generation ACI

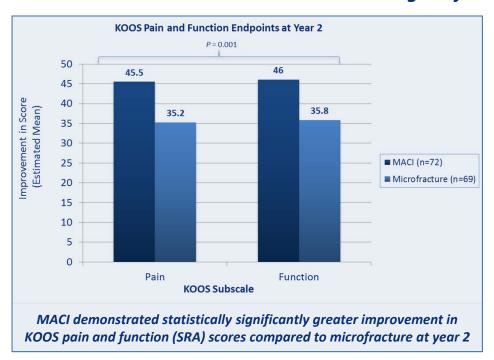
- Less invasive ACI
- Easier administration
- Eliminates periosteal harvest and sutures
- Significant reduction in surgical time
- Uniform distribution of cells
- Improved post-operative course

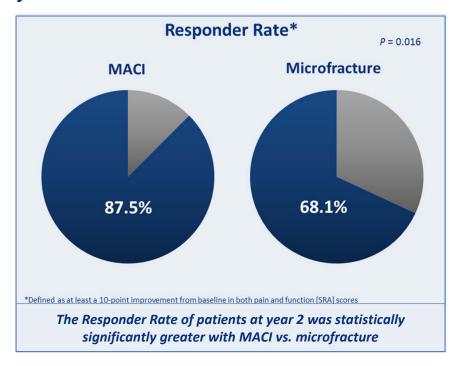




# SUMMIT (<u>Superiority of MACI Implant Versus</u> <u>Microfracture Treatment</u>) Clinical Study Results<sup>1</sup>

# Overall efficacy data support a long-term clinical benefit from the use of MACI in patients with cartilage defects of the knee



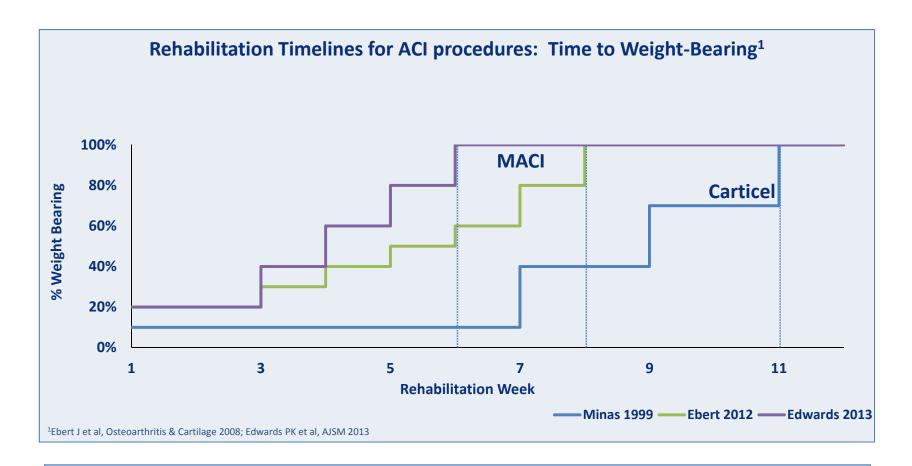


- In a three year follow-up study, the mean two-year KOOS pain and function scores remained stable for the additional three-year period
- The most frequently occurring adverse reactions (>5%) for MACI were arthralgia, tendonitis, back pain, joint swelling and joint effusion





## **MACI** Rehabilitation Protocol

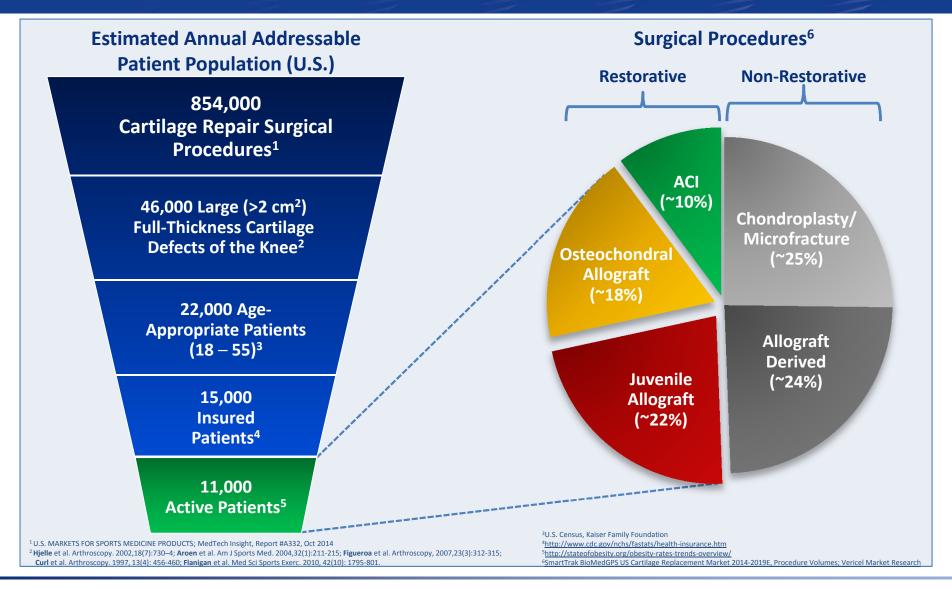


Published MACI rehabilitation protocols achieve full weight-bearing in 6-8 weeks compared to 10-12 weeks for published Carticel rehabilitation protocols





# Large Addressable Cartilage Repair Market for MACI







## **MACI Strategic Investments**

- Expanded Commercial and Medical Affairs Team
  - Expanded Sales Regions and Area Sales Directors from two to four
  - Expanding Sales Territories from 21 to 28
  - Adding a dedicated in-house sales trainer, Market Access Director, and MSL to support expected growth in customer base
- Enhanced Patient and Customer Support Programs
  - Train-the-Trainer Program
    - Leading European and Australian KOLs with extensive MACI experience are conducting speaker training and leading a bioskills lab with top U.S. KOLs
  - In-person and state-of-the-art online surgeon training tools and apps
  - MACI.com website
  - MyCartilageCare healthcare provider website
  - Interactive visual aid and resources digital tool box
  - Payer support Account Executive Team, Budget Impact Model





# MACI Healthcare Provider and Patient Support Tools





### **HCP Brochure**



### **Patient Brochure**



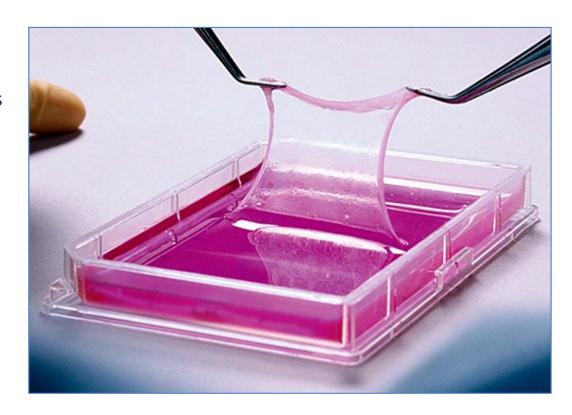




# **Epicel Overview**

# Epicel is a permanent skin replacement for full thickness burns ≥ 30% of total body surface area

- Only FDA-approved autologous epidermal product available for large total body surface area burns
- Important treatment option for severe burn patients where little skin is available for autografts
- Approved as a Humanitarian Use Device in the United States
- FDA approved HDE Supplement to revise label to specifically include pediatric patients (February 2016)







# **Epicel Production and Administration**

Biopsy Harvest

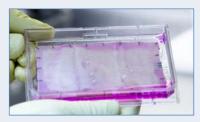
## **Epicel Production**



**Keratinocyte Expansion** 



**Epicel Sheet** 



**Epicel Graft** 

## **Epicel Delivered**



## **Epicel Administration**



**Graft Removal** 



**Grafts Applied** 



Takedown Procedure



New Skin Exposed





# Revised Epicel Label Will Enable Continued Growth

# Epicel® (cultured epidermal autografts) HDE# BH990200

Epicel may now be sold for profit on up to 360,400 grafts per year (>50X 2015 volume)

#### **Directions for Use**

**HUMANITARIAN DEVICE**: Authorized by Federal law for use in adult and pediatric patients who have deep dermal or full-thickness burns comprising a total body surface area greater than or equal to 30%. **Epicel**<sup>®</sup> may be used in conjunction with split-thickness autografts, or alone in

Vericel may now communicate the probable survival benefit of Epicel in all age groups to physicians

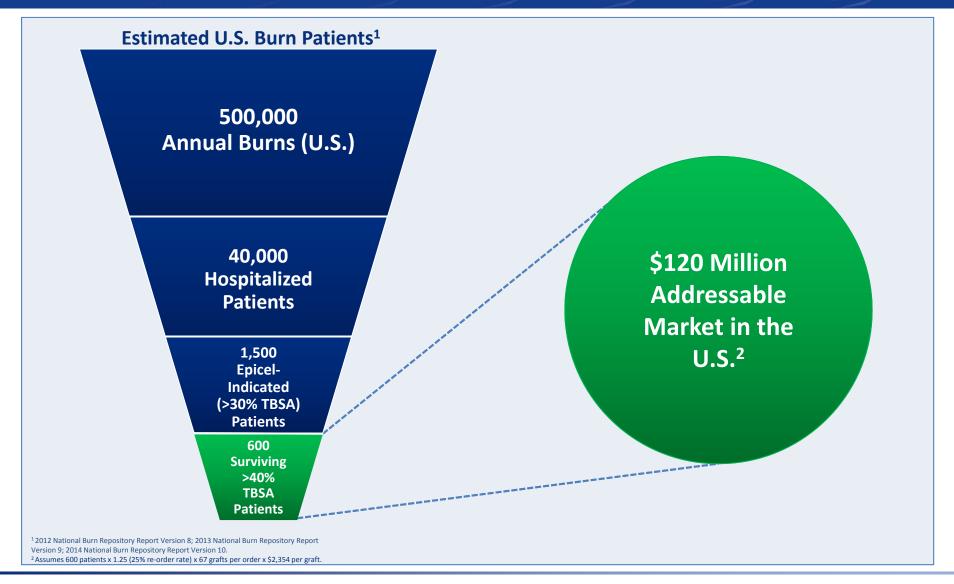
### **CLINICAL STUDIES**

The probable benefit of Epicel<sup>®</sup>, mainly related to survival, was demonstrated in two Epicel databases and one physician-sponsored study, as shown in Table 3, Table 4, and Table 5.





# Large Addressable Burn Therapy Market for Epicel







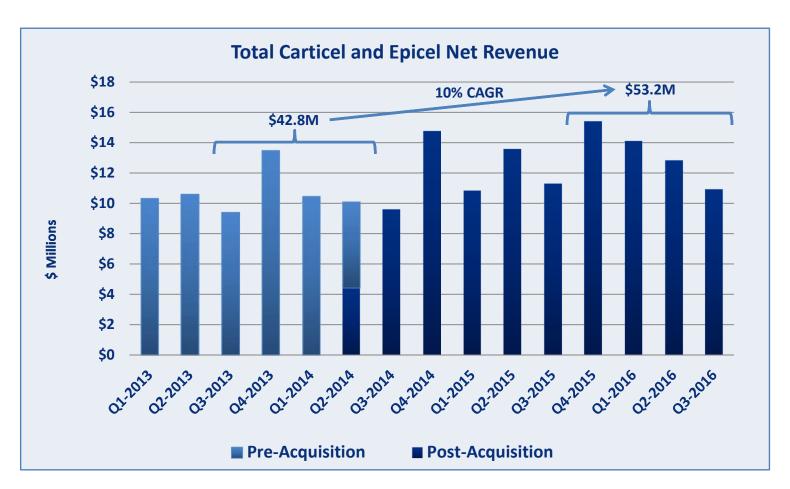
## **Epicel Strategic Investments**

- Expanded Commercial and Medical Affairs Team
  - Expanded to five sales representatives and a dedicated Regional Sales Director
  - Hired a dedicated MSL
- Enhanced Patient and Customer Support Programs
  - Comprehensive peer-to-peer programs including Advisory Boards, Fellowship Programs and Medical Programs
  - Enhanced training and reimbursement support
  - Increased presence through sponsorships, publications, and public relations campaigns





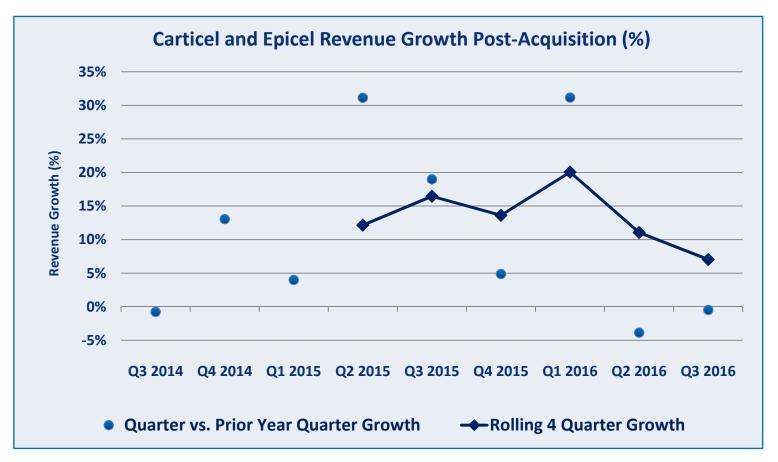
# Strong Total Revenue Growth In the Core Commercial Business



- LTM revenue = \$53.2 million
- 10% CAGR in revenue since the acquisition of Carticel and Epicel



# Strong Total Revenue Growth Rate In the Core Commercial Business



- Variable growth rate quarter vs. prior year quarter growth ranges from -4% to 31% due to seasonality and specialty biologics business model
- Rolling four-quarter growth rate between 7% and 20%



## Robust Financial Results Post-Acquisition

Condensed Quarterly Income Statement								
	Q4 2014	Q1 2015	Q2 2015	Q3 2015	Q4 2015	Q1 2016	Q2 2016	Q3 2016
Revenues	\$14,706	\$10,849	\$13,590	\$11,309	\$15,420	\$14,108	\$12,823	\$10,929
Cost of Product Sales	6,752	5,568	6,901	6,772	7,229	6,560	7,302	6,856
Gross Profit	7,954	5,281	6,689	4,537	8,191	7,548	5,521	4,073
R&D	5,794	4,377	3,369	3,740	7,404	3,536	4,057	3,443
SG&A	4,506	5,476	5,585	5,674	5,744	6,004	6,448	7,010
<b>Total Operating Expenses</b>	10,300	9,853	8,954	9,414	13,148	9,540	10,505	10,453
Loss from Operations  Add back one-time adjustmen	\$ (2,346)	\$ (4,572) -	<u>\$ (2,265)</u>	\$ (4,877) 1,037	\$ (4,957) <sup>2</sup> 4,536	\$ (1,992)	\$ (4,984)	\$ (6,380)
Adjusted Loss from Operations 1) Verigen Shareholder Payment	\$ (2,346)	\$ (4,572)	\$ (2,265)	\$ (3,840)	\$ (421)	\$ (1,992)	<u>\$ (4,984)</u>	\$ (6,380)

Select Balance Sheet Items		
		30-Sep-16
Cash	\$	8,880
<b>Total Current Assets</b>	\$	21,099
Total Assets	\$	28,022
Total Current Liabilition	\$	15,360
<b>Total Liabilities</b>	\$	16,629

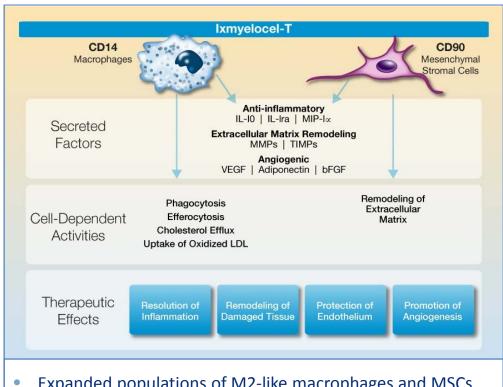
2) MACI BLA and Epicel HDE Regulatory Consulting Expenses

- Margins are expected to continue to improve as volumes increase given low marginal costs and existing capacity
- Operating profit expected to increase as ixmyelocel-T development costs decline
  - \$7.4 million of internal and external R&D expense in the trailing four quarters were due to ixCELL-DCM study costs
- Cash balance of \$8.9 million as of September 30, 2016
  - Subsequent ~\$20 million financing closed on December 21, 2016
  - \$10 million A/R facility and \$10 million term loan with SVB and MidCap Financial (\$6 million utilized as of Q3 2016)



Gross margins of 47% for trailing four quarters

# Ixmyelocel-T is a Highly Differentiated Multicellular Therapy With a Scalable GMP Manufacturing Platform



- Expanded populations of M2-like macrophages and MSCs
- Multiple biological activities that promote tissue repair and regeneration

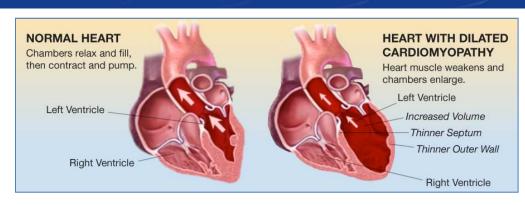




- Automated, Fully-Closed GMP System
  - Single-use disposable bioreactor cassette
- Scalable Modular Expansion
  - Enables COGS < 10% at commercial scale</li>



# Ixmyelocel-T for Treatment of Advanced Heart Failure Due to Ischemic DCM – Fast Track Program Designation



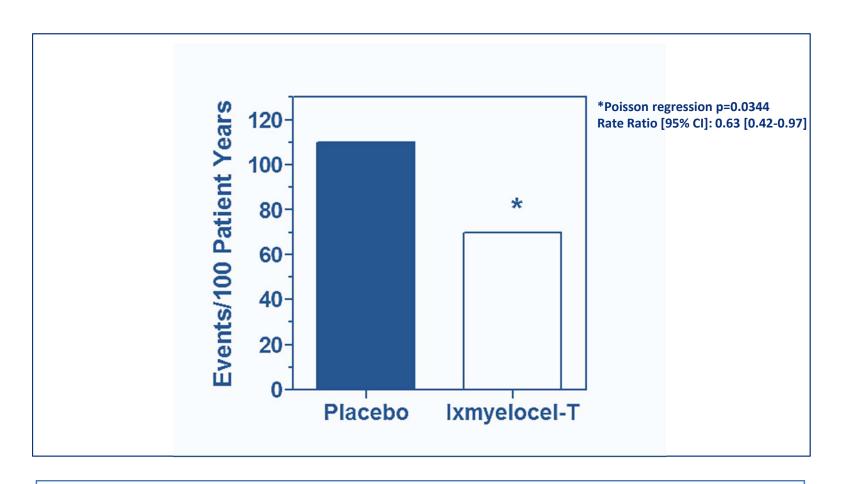
#### **Patient Profile**

- Majority of patients that are refractory to medical therapy have ischemic dilated cardiomyopathy (DCM) (~150,000 patients)
- Maximized Rx and device therapy and typically no longer candidates for revascularization procedures
- Only remaining options are LVAD or heart transplant

Phase 2b ixCELL-DCM Study Design				
Objectives	<ul> <li>To evaluate the efficacy, safety and tolerability of ixmyelocel-T compared to placebo in patients with heart failure due to ischemic DCM</li> </ul>			
Patients	<ul> <li>Diagnosis of ischemic DCM according to WHO criteria</li> <li>Males and females, age 30-86</li> <li>LVEF ≤ 35%</li> <li>NYHA class III or IV heart failure</li> </ul>			
Design	<ul> <li>Multicenter, randomized (1:1), double-blind, placebo-controlled phase 2b study</li> <li>108 patients at approximately 35 sites in the US and Canada</li> <li>Administration via catheter injection into the left ventricular endocardium using the NOGA® Myostar® injection catheter (Biosense Webster)</li> </ul>			
Primary Endpoint	<ul> <li>Number of all-cause deaths, cardiac hospitalizations, and unplanned outpatient/ emergency department visits for IV treatment of acute worsening heart failure over 12 months</li> </ul>			
Status	Results presented at the Late-Breaking Clinical Trials session at ACC and published in <i>The Lancet</i>			



# ixCELL-DCM Clinical Trial: Primary Efficacy Endpoint

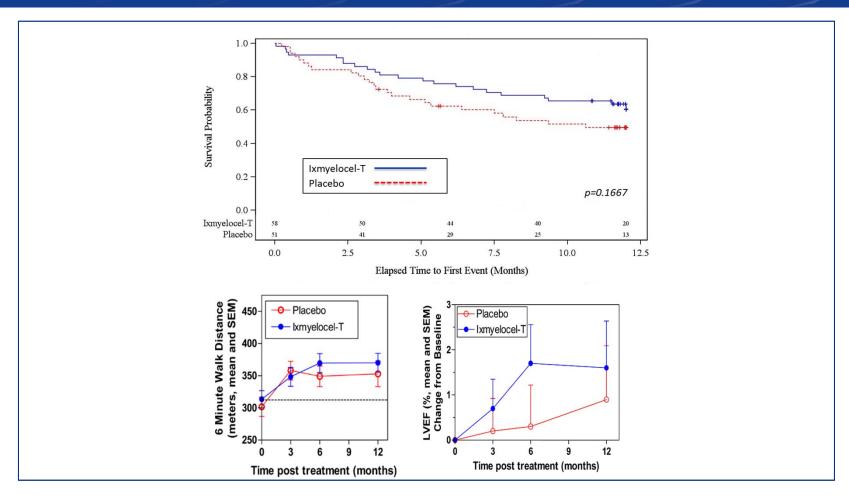


Patients treated with ixmyelocel-T had a 37% reduction in events compared to placebo





# Phase 2b ixCELL-DCM Clinical Trial Results: Selected Secondary Efficacy Endpoints



Secondary endpoints favored ixmyelocel-T, but differences were not significant





## ixCELL-DCM Trial Results Published in The Lancet



## Ixmyelocel-T for patients with ischaemic heart failure: a prospective randomised double-blind trial



Amit N Patel\*, Timothy D Henry\*, Arshed A Quyyumi, Gary L Schaer, R David Anderson, Catalin Toma, Cara East, Ann E Remmers, James Goodrich, Akshay S Desai, David Recker, Anthony DeMaria, for The ixCELL-DCM Investigators

#### Summary

Background Ixmyelocel-T is an expanded, multicellular therapy produced from a patient's own bone marrow by Published Online selectively expanding two key types of bone marrow mononuclear cells: CD90+ mesenchymal stem cells and CD45+ CD14+ auto-fluorescent+ activated macrophages. Early phase clinical trials suggest that intramyocardial delivery of ixmyelocel-T might improve clinical, functional, symptomatic, and quality-of-life outcomes in patients with heart failure due to ischaemic dilated cardiomyopathy. We aimed to assess the safety and efficacy of catheter-based transendocardial injection of ixmyelocel-T cell therapy in patients with heart failure and reduced ejection fractions.

April 4, 2016 http://dx.doi.org/10.1016/ 50140-6736(16)30137-4 http://dx.doi.org/10.1016/





# Vericel Investment Highlights

Vericel Investment Highlights					
Robust Specialty Biologics Business	<ul> <li>Fully integrated specialty biologics business with strong revenue growth and expanding gross margins</li> <li>Total Carticel® and Epicel® trailing 12-month revenues of \$53.2 million as of Q3 2016         <ul> <li>10% CAGR in revenue since acquisition</li> </ul> </li> </ul>				
Near- and Long-Term Growth Drivers	<ul> <li>MACI® BLA approved by the FDA on December 13, 2016 – potential to significantly expand cartilage repair franchise</li> <li>Epicel HDE supplement approved in February 2016 – revised label includes pediatric patients and probable survival benefit; allows Epicel to be sold for profit</li> <li>Ixmyelocel-T Phase 2b ixCELL-DCM trial for treatment of advanced heart failure due to ischemic DCM met primary endpoint – Fast Track designation granted February 16, 2017</li> </ul>				
Strong Shareholder Base	<ul> <li>Closed \$20 million financing in December 2016</li> <li>Participation by leading institutional healthcare investors</li> </ul>				
Experienced Management Team	<ul> <li>Strong track record of developing and commercializing products in the U.S.</li> <li>Deep experience in restructuring and integrating acquired businesses</li> </ul>				



# **Appendix**



# Management Team with Deep Operations and Commercialization Experience

## **Management Team**

### Nick Colangelo – President & CEO (March 2013)

- More than 20 years of executive management and corporate development experience
- Nearly a decade with Eli Lilly, including serving as Director of Strategy and Business Development for Lilly's Diabetes Product Group and founding Managing Director of Lilly Ventures
- Extensive experience in the acquisition, development and commercialization of therapies to treat fibrovascular, metabolic and CV diseases

#### Gerard Michel – Chief Financial Officer and Vice President, Corporate Development (June 2014)

- More than 20 years in the life science industry including large pharma (Lederle Labs, Wyeth Labs), biotech (NPS Pharmaceuticals, Biodel) and management consulting (Booz Allen) with meaningful experience across all major functional and therapeutic areas
- Raised significant amount of capital via strategic, equity, debt, and royalty deals

#### **Daniel Orlando – Chief Operating Officer (August 2012)**

- More than 20 years of sales, marketing, and business development experience, most recently serving as Vice President of business development for North and South America at Takeda
- Extensive commercial experience in cardiovascular, diabetes and metabolic disease areas
- Original brand director for Actos

#### David Recker, M.D. - Chief Medical Officer (April 2014)

- More than 20 years of drug development experience, most recently as Senior Vice President for Clinical Science at Takeda Global R&D
- Responsible for multiple programs in a variety of therapeutic areas, including cardiovascular, diabetes, and metabolic disease areas
- Numerous successful regulatory filings throughout the world



# Vericel Capitalization Table

Capitalization (as of January 31, 2017)	Shares
Common Stock	31,594,972
Series B Preferred Stock – Common Equivalents*	615,400
Accumulated but Undeclared Series B-1 Stock Dividends*	455,308
August 2013 Warrants	724,950
September 2016 Warrants	117,074
Options Outstanding	3,403,625
Fully Diluted Shares Outstanding	<u>36,911,329</u>

<sup>\*</sup> The Series B preferred stock is convertible to common shares on March 9, 2017. Series B preferred stock dividends will accrue through March 9, 2017 and will total 478,492 common shares upon conversion.

