

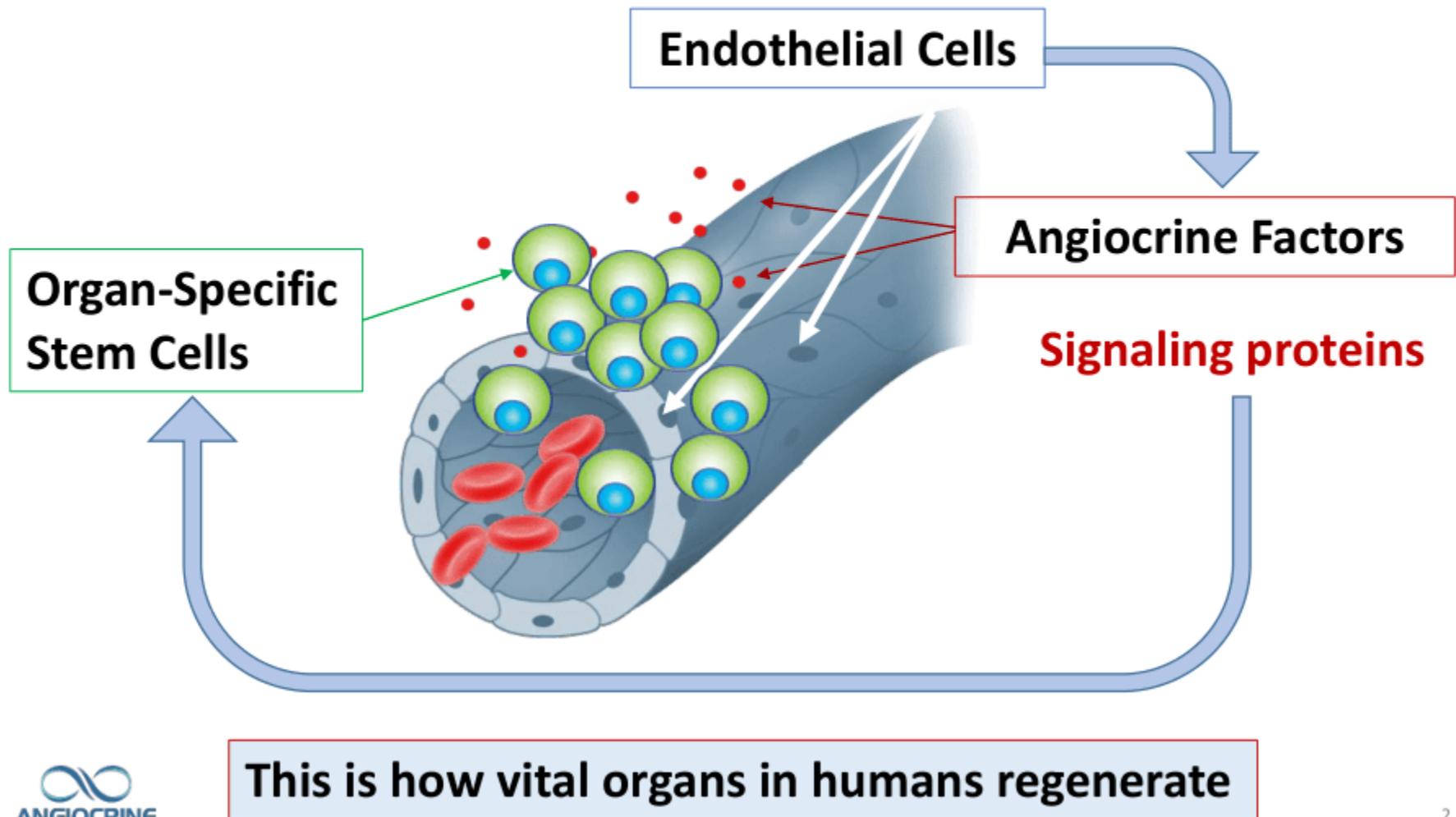


ANGIOCRINE
BIOSCIENCE

Investor Update
May 4th 2017



Fact: Endothelial Cells Instruct Stem Cells



Uniquely Angiocrine Bioscience

E-CEL™

Injectable engineered human endothelial cells that can orchestrate regeneration of vital organs

Ψ-CEL™

Create **your own stem cells** from your own endothelial cells obtained via skin or fat samples

No other company has technologies like ours

Rapid Progress Over the Last Year

- 1) Awarded non-dilutive \$3.8M grant for pre-clinical development
- 2) Created two clinical therapeutic candidates
- 3) Filed with FDA 1st Investigational New Drug (IND) application to enter clinic
- 4) New IP in cellular biopharmaceutical and stem cell gene therapy
- 5) Established full R&D capabilities in San Diego



E-CEL™ Clinical Development

*Focus on Transforming
Hematology & Oncology*



1. E-CEL Stem-Cell Gene Therapy

- Goal: Cure genetic blood diseases(e.g., Sickle Cell Anemia)
- Clinical stage: H2 2017 (1st trial, Leukemia with high-risk genetic characteristics)

2. E-CEL Cellular Therapy

- Goal: Accelerate recovery from radiation/chemotherapy
- Clinical stage: H1 2018 (1st trial, various cancers)

Substantial Global Markets

Gene Therapy Market

- **Genetic diseases** (e.g. *Sickle Cell Anemia, Primary Immuno-Deficiency, etc.*) are responsible for a **heavy loss of life.**¹
- **> 6,000 known single-gene defect disorders**²
- **“worth USD 11 billion by 2025”**³
- **“an area of important future growth”** (GlaxoSmithKline)⁴

Cancer Care Market

- **40% of adults will be diagnosed with cancer** during their lifetimes⁵
- **1,685,210 new US cancer cases diagnosed** in 2016⁵
- **Significant number of patients will receive high-dose chemotherapy and/or radiation treatment**



¹www.who.int/genomics/public/geneticdiseases/en/index2.html

²www.medicinenet.com/genetic_disease/article.htm

³www.prnewswire.com/news-releases/gene-therapy-market-2015--2025-300082822.html

⁴ MIT Technology Review, May 16th 2016, *Gene Therapy's First Out-and-Out Cure is Here.*

⁵ Cancer Statistics - National Cancer Institute 2016

E-CEL Stem-Cell Gene Therapy Program

CURE



Clear US FDA Regulatory Path

E-CEL Stem-Cell Gene Therapy

CURE



First-In-Human Clinical Trial (Phase 1b)

- Safety of stem cells expanded in culture with E-CEL cells
- Stem cells are not gene corrected and obtained from donor umbilical cord blood (allogeneic)
- Patients with blood/marrow cancer with high-risk genetic profile

Second Clinical Trial (Phase 2)

- Safety and efficacy of stem cells expanded in culture with E-CEL cells
- Stem cells are gene corrected and obtained from patient's bone marrow (autologous)
- Patients with genetic blood disease (e.g., sickle cell anemia)

First-in-Human Clinical Trial

E-CEL Stem-Cell Gene Therapy

CURE



Financial Support from:



State of California Stem Cell Research and Cures Initiative publicly funded program to advance scientific discoveries in stem cell biology

- **Pre-clinical grant award (April, 2016) - \$3.8M**
 - ✓ Covered 80% of all pre-clinical development costs
- **Clinical grant application – (Q2, 2017) – up to \$5M**
 - ✓ Covers 70% of Phase1b Clinical trial costs

Therapeutic focus:

- Rebuild new blood and immune system by transplanting E-CEL cord blood stem cells (transplantation) in high-risk leukemia patients who cannot be cured by chemotherapy or other donors

Key milestones:

- Submitted IND to enter first clinical trial in 2H17
- Clinical supply material manufactured in GMP facility for trial



Phase 2 Clinical Trial

E-CEL Stem-Cell Gene Therapy

CURE



New York's publicly funded program to advance scientific discoveries in stem cell biology

- **Award Amount - \$15.7M**

- **Award Recipients:**

- Consortium led by Angiocrine Scientific founder – Shahin Raffi, MD (Weill Cornell Medical) and collaborator Michel Sadelain, MD (Memorial Sloan Kettering Cancer Center)



Weill Cornell Medical College



Memorial Sloan Kettering Cancer Center.

- **Disease Focus: Sickle Cell Anemia**

- **Purpose:**

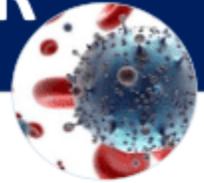
- Combine Angiocrine's E-CEL Technology with Sloan Kettering's gene correction technology to develop a curative therapy for sickle cell disease
 - Funds research, pre-clinical development and IND

Angiocrine retains commercial rights to E-CEL



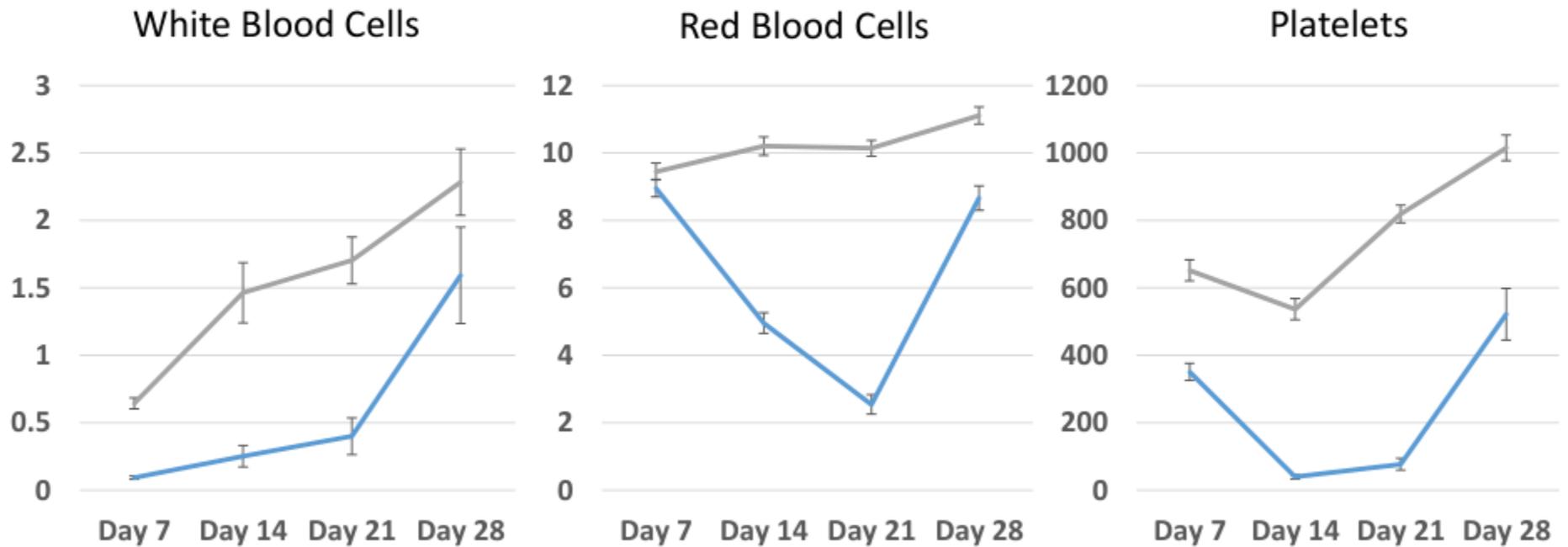
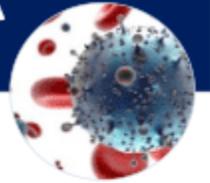
E-CEL Cellular Therapy Program

RECOVER



E-CEL Infusion Accelerates Blood & Immune System Recovery

RECOVER



— Control

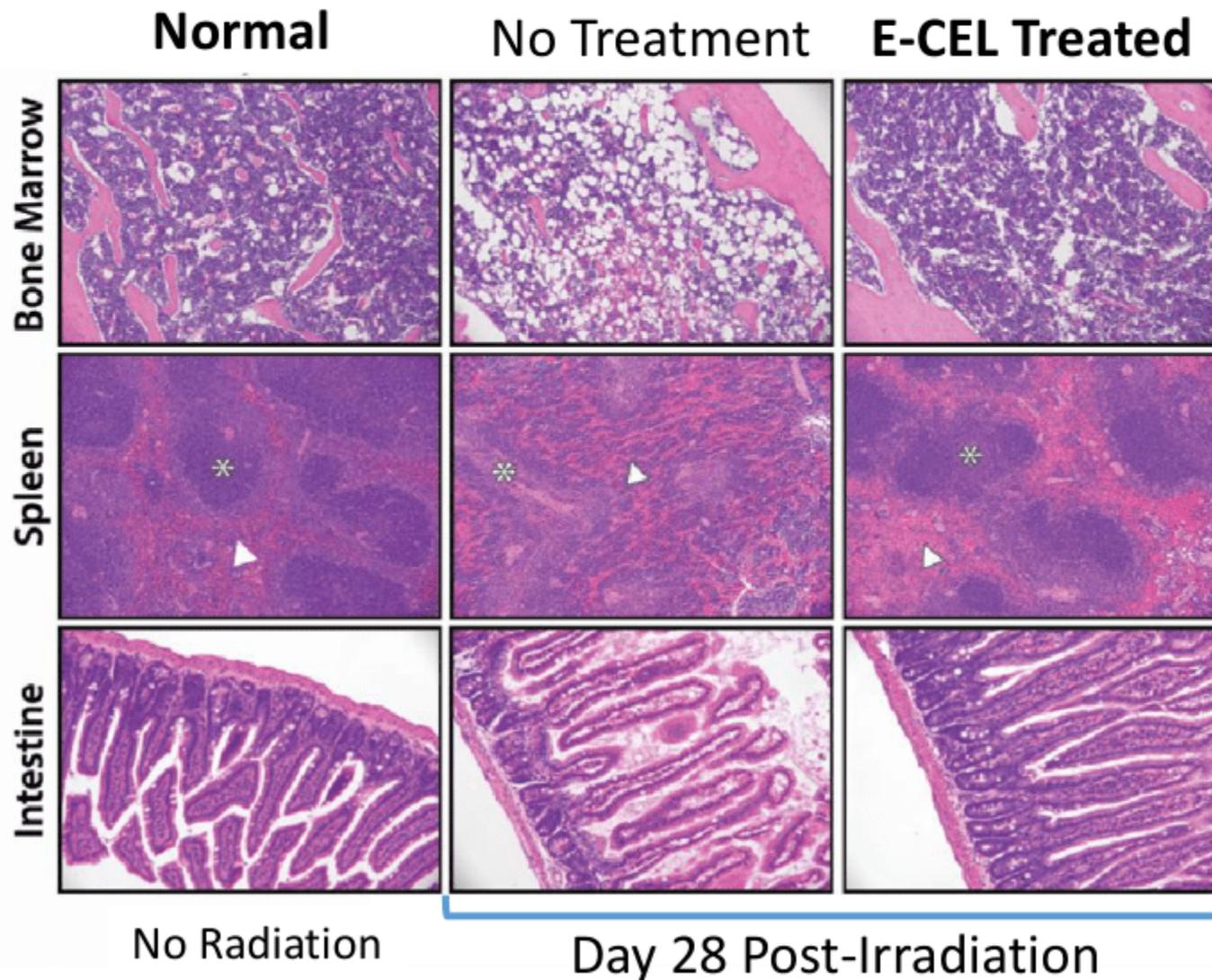
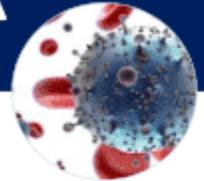
— Treated with E-CEL



Immunocompetent C57BL/6 mice – Treatment Day +1 post-LD50 irradiation

And Also... Systemic Recovery

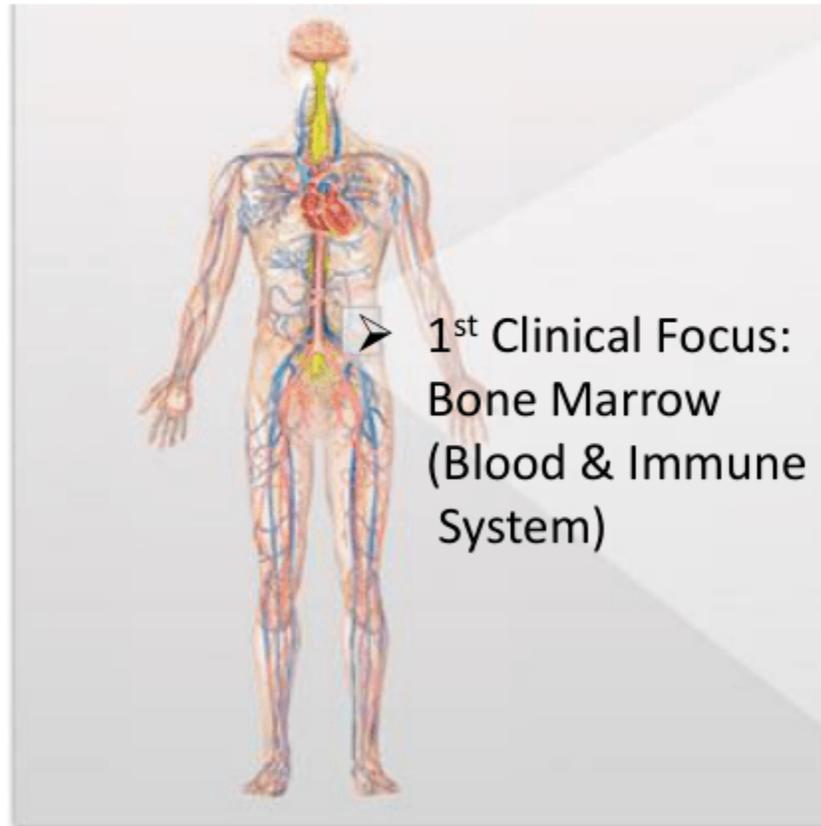
RECOVER



Multiple Clinical Applications of E-CEL™

Applies to virtually all organ regeneration

- Brain & Spine
- Heart
- Lungs
- Liver
- Kidneys
- Muscles & Tendons



Angiocrine's Active Research Pipeline

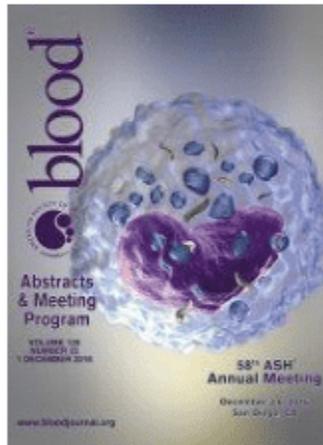
Partnering with Experts – Resource Efficient

Program	Aim	Collaborator
Ψ-CEL™ Stem Cells	Produce one's own blood stem cells from own fat	 Weill Cornell Medical College
Neural E-CEL	Restore function in spinal cord injury	  SPINAL CORD RESEARCH CENTER Drexel University · College of Medicine
Lung E-CEL	Restore function in chronic lung disease	 Weill Cornell Medical College
Liver E-CEL	Repair without fibrosis in chronic liver disease	 Weill Cornell Medical College
Tendon/Muscle E-CEL	Accelerate healing, strengthen tendon repair	 HOSPITAL FOR SPECIAL SURGERY
E-CEL Gene Therapy	Treat and cure HIV patients with Lymphoma	 City of Hope RESEARCH · TREATMENT · CURES

Angiocrine Research: Ψ -CEL Technology

Create your own stem cells from your skin or fat sample

Transformative potential for longevity and rejuvenation science



Direct Conversion of Adult Endothelial Cells into Immune-Competent Long-Term Engraftable Clinically Scalable Hematopoietic Stem Cells: Pathway to Therapeutic Translation

Lis R et. al., Blood 2016 128:372;



Weill Cornell Medical College

NATURE | VOL 511 | 17 JULY 2014

Reprogramming human endothelial cells to haematopoietic cells requires vascular induction

Vladislav M. Sandler¹, Raphael Lis^{1,2}, Ying Liu¹, Alon Kedem^{1,2}, Daylon James^{1,2}, Olivier Elemento³, Jason M. Butler¹, Joseph M. Scandura⁴ & Shahin Rafii¹

nature



Series A-1 Equity Financing

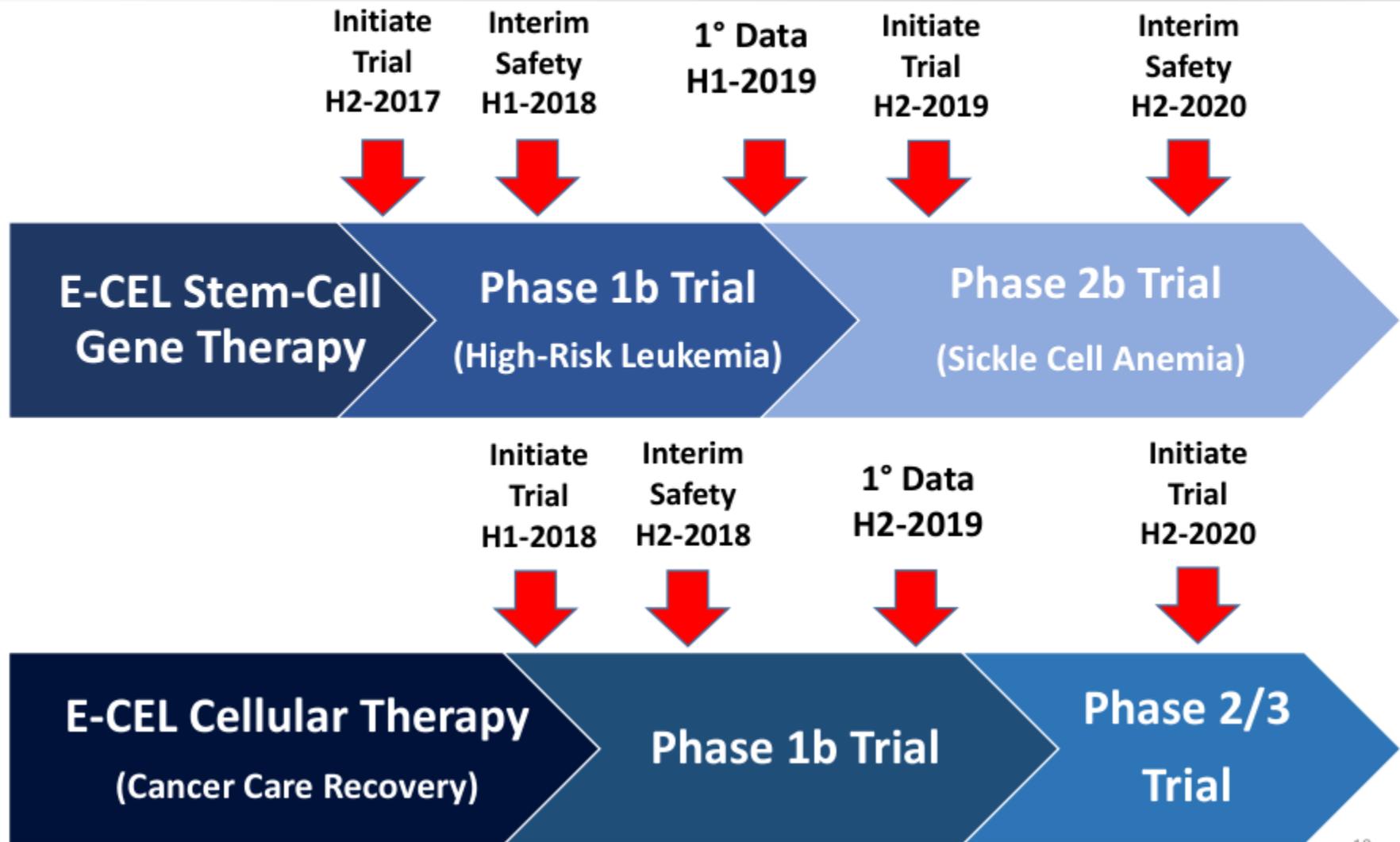
Pre-Money Valuation: \$27M

Minimum raise: \$4M

Maximum: \$8M

Series A-1	Proceeds towards objectives:
\$4M	'matching' funds to obtain additional \$5M CIRM clinical grant for 1 st E-CEL study & complete trial
→ \$6M	+ advance E-CEL chemo/rad recovery to 2 nd IND + in-license gene therapy vector (e.g., sickle cell)
→ \$8M	+ complete 2 nd IND (cancer chemo/rad treatment recovery program) & apply for 2 nd \$5M CIRM grant + initiate pre-clinical gene therapy studies

Anticipated Inflection Points



Angiocrine Bioscience Investment Thesis

1. Prior to value inflection points

- Transition to clinical stage
- Two clinical assets
- Expanding pipeline & partnerships

2. Risk-mitigated:

- Multiple shots to succeed
- Pre-clinical toxicology done on E-CEL
- Thoroughly vetted by experts (grants, publications)
- Value in robust IP

3. Leveraged: opportunity for more non-dilutive grants

4. Experienced Leadership: solid records, prior exits

THANK YOU!

Contacts:

Paul Finnegan, MD, MBA - CEO

John Jaskowiak - VP, Corporate Development

*E-CEL™ Therapies
Ψ-CEL™ Technology*

*Transforming
Hematology and Cancer Care
via the powers of
Engineered Endothelial Cells*

April 2017

