CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

*The state stem cell agency*

# Tools and Technology III (TNT3)

**Presentation to ICOC**

**Agenda Item 7**

**January 29, 2015**

**Lila Collins**

# Overview

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- TNT Program and TNT3 RFA Introduction
- GWG Recommendations
- Programmatic Review
- CIRM Team Recommendations

# CIRM's Tools and Technology Program

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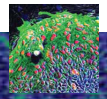
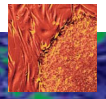
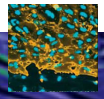
- TNT1, 2008, closed, more basic focus, 23 awards, $19M
- TNT2, 2010, closing in Q2, translational focus, 19 awards, $33M
- TNT3, 2013, translational focus, for consideration today



# TnT 3: Goal and Scope

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**Goal**: to overcome translational bottlenecks to stem cell therapies

**Responsive Projects**

- Address translational bottlenecks to human stem cell therapies, broadly applicable and shared
- Create and test novel tools and technologies
- Optimize, standardize or scale up existing tools or technologies

# TNT3 Program Information

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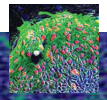
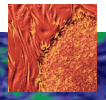
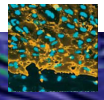
- Collaborative, multidisciplinary
- $35M total approved budget, approximately 20 grants
- 3 yrs, up to $900k justifiable direct project costs; up to $1.2M justifiable direct project cost for large animal models



# Review Criteria Highlights

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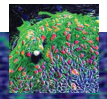
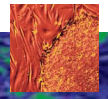
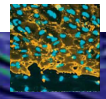
**Significance and Rationale**: Will the tool or technology improve our ability to bring stem cell therapies to patients? Will it have a major impact?

**Feasibility and Experimental Design** Is everything in place to perform the research? Is the plan logical and sound?

**Qualifications of PI and Team** Does the team have an appropriate and relevant track record track record to successfully lead the project? Are key collaborations secured?

**Responsiveness to RFA's Goals**



# Scoring Instructions to GWG

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- Tier 1, scores of 75 and above = recommended for funding, meritorious
- Tier 2, scores of 65-74 = moderate quality, or consensus on merit not reached, *may be suitable for programmatic consideration*
- Tier 3, scores of 1-64 = not recommended for funding, not meritorious
- GWG used the full scoring scale

# RFA Priorities for Programmatic Consideration

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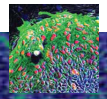
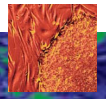
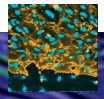
- Clinically compatible technologies to achieve cell survival, engraftment and integration

- For indications where large animal modeling is **critical**
  - Technologies to enable long-term human xenograft retention
  - Therapeutically relevant cell types from pluripotent stem cells of large animal model species

- Methods or reagents to enable clinical imaging and tracking of human stem cells at high sensitivity



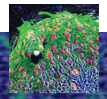
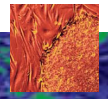
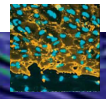




# RFA Priorities - Continued



- Xenobiotic free reagents for stem cell production processes
- Methods to expand human HSC and/or generate reconstituting HSC from human pluripotent stem cells
- Application of clinically compatible nanotechnologies for human stem cell modulation, in vivo monitoring or elimination
- Where it increases the likelihood of the projects success, applications that provide additional funding



# GWG Review Perspective

# CIRM Team Recommendation Process

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- Noted a cluster of 4 tier 2 applications, median score 75
- Fund recommendations from 9 of 15 reviewers
- CIRM recommends 3 for funding

# 7678, “A Small Molecule Tool For Reducing The Malignant Potential…”, score 74

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- CIRM Recommendation = FUND
- Technology = a small molecule to selectively remove/kill residual undifferentiated PSC from PSC-derived therapies ex vivo to address the safety bottleneck.
- Highlights
  - Teratoma risk is a significant bottleneck in the translation of PSC derived stem cell therapies
  - A small molecule could prove more time and cost effective than sorting approaches
  - No active grants addressing this translational challenge in our portfolio

# 7899 “Development of 3D Bioprinting Techniques using hESC-CM for Cardiac…” score,73

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- CIRM Recommendation = FUND
- Technology = 3D printing of vascularized cardiac tissues, to address the engraftment bottleneck
- Highlights
  - 3D printing is potentially transformative technology for production
  - The PI is a leader in the field
  - No active grants utilizing 3D printing in our portfolio

# 7838, “Development of a scalable, practical and transferable GMP-compliant…” score 72

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- CIRM Recommendation = FUND
- Technology = bag suspension culture and differentiation, to address manufacturing bottleneck
- Highlights
  - Builds upon prior CIRM TNT funded GMP process development success by the lead investigator
  - Complements an active CIRM development award
  - No active grants utilizing the proposed bioreactor in our portfolio

# 7981, “Multimodal technology for non-destructive characterization…”, score 72

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- CIRM Recommendation = DO NOT FUND
- Technology = multimodal imaging tool combining light and ultrasound methods
- Highlights
  - Tier 1 application, RT3-07879, involves overlapping leadership and will develop the same imaging technology with a different test article.

# Programmatic Review