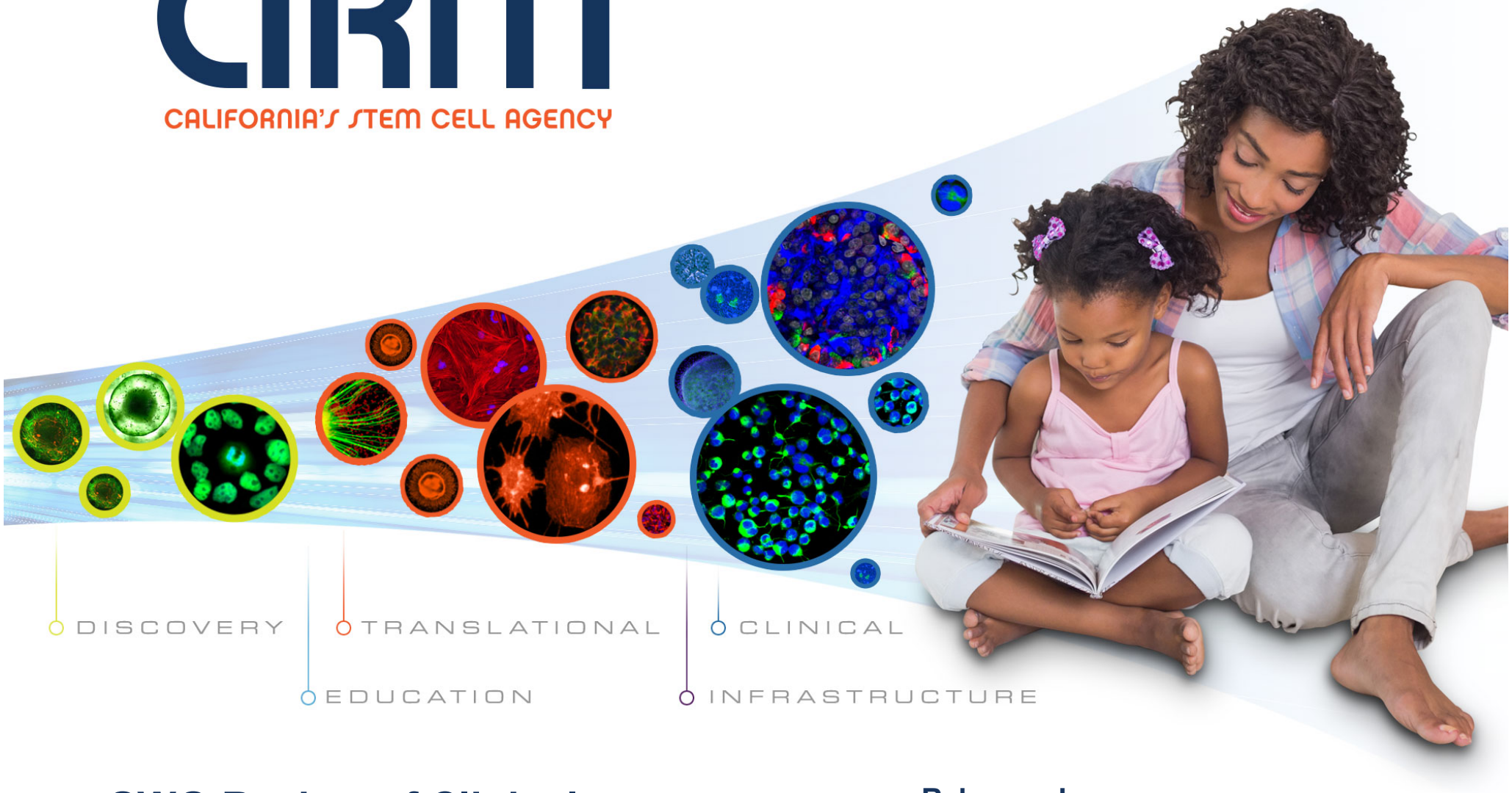
AGENDA ITEM #9
ICOC MEETING
March 16, 2016

CIRM
CALIFORNIA'S STEM CELL AGENCY

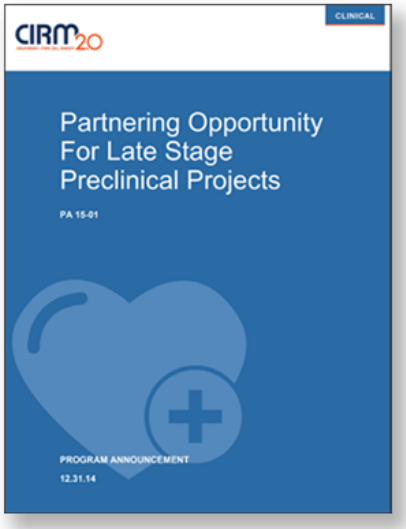


GWG Review of Clinical Program Applications

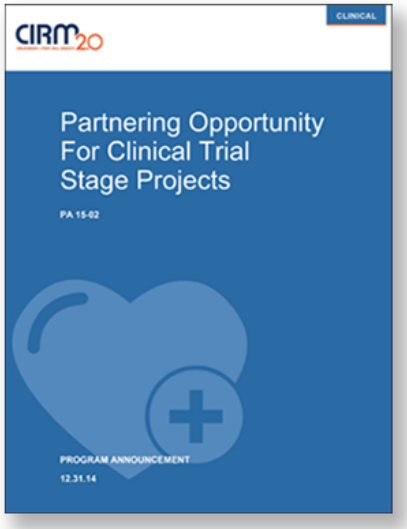
Rebecca Jorgenson
Senior Science Officer
Portfolio Development and Review

Clinical Stage Programs

CLINICAL STAGE



CLIN 1



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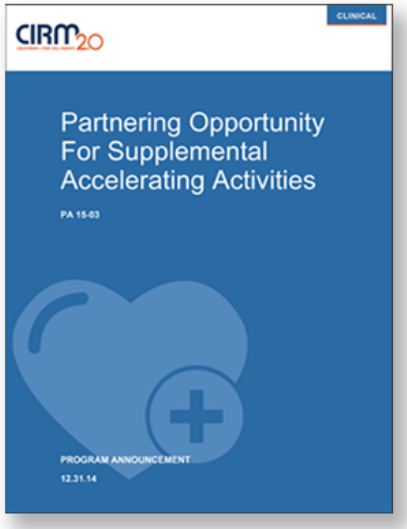

CLIN 3

CIRM 2.0
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Scoring System for 2.0 Applications

- Score of “1”

  Exceptional merit and warrants funding.

- Score of “2”

  Needs improvement and does not warrant funding at this time but could be resubmitted to address areas for improvement.

- Score of “3”

  Sufficiently flawed that it does not warrant funding and the same project should not be resubmitted.

Applications are scored by all scientific members of the GWG with no conflict.

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CLIN2-08334: Phase 2 Clinical Trial for Cardiomyopathy in Muscular Dystrophy Patients

Therapy: Allogeneic Cardiosphere-Derived Cells (CAP-1002)

Indication: Duchenne Muscular Dystrophy (DMD) Cardiomyopathy

Goal: Complete a randomized, open label Phase 2 clinical trial to test safety and efficacy of CAP-1002 in patients with cardiomyopathy secondary to DMD.

Major Proposed Activities:

- Manufacture CAP-1002 in quantities sufficient to treat all subjects enrolled in the trial.
- Enroll and treat all subjects per the clinical protocol.

Funds Requested: $3,376,259 ($2,254,032 Co-funding)

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CLIN2-08334: Phase 2 Clinical Trial for Cardiomyopathy in Muscular Dystrophy Patients

Budget Review: Pass

GWG Score: 1* Exceptional merit, warrants funding

- Votes for score of 1: **12**
- Votes for score of 2: **1**
- Votes for score of 3: **0**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: $3,376,259

*2nd revision score - original “2” (2-7-4); 1st revision “2” (6-9-0)

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GWG Vote on Review Process

1. All members: “The review was scientifically rigorous, there was sufficient time for all viewpoints to be heard, and the scores reflect the recommendation of the GWG.”

2. ICOC patient advocate members: “The review was carried out in a fair manner and was free from undue bias.”

All members voted unanimously in favor of 1 (19-0)

ICOC members voted unanimously in favor of 2 (6-0)

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CLIN1-08363: Preclinical Development of a Gene Therapy Approach for Artemis-Deficient SCID

Treatment: Autologous CD34+ hematopoietic stem cells (HSC) from Artemis-deficient severe combine immunodeficiency (ART-SCID) patients modified by gene therapy to express a corrected copy of the Artemis gene.

Indication: Patients with ART-SCID lacking a matched sibling transplant donor or who have failed allogeneic transplant.

Goal: Complete preclinical research activities and submit an IND to conduct a subsequent clinical trial.

Major Proposed Activities:

- Manufacture sufficient preclinical vector for toxicity and efficacy studies and clinical grade vector for the subsequent clinical trial.
- Complete nonclinical toxicity studies and demonstrate ability to manufacture transduced human cells at clinical scale.
- Complete nonclinical efficacy studies.

Funds Requested: $4,268,865 ($0 Co-funding)

CIRM
CALIFORNIA'S STEM CELL AGENCY

CLIN1-08363: Preclinical Development of a Gene Therapy Approach for Artemis-Deficient SCID

Budget Review: Pass

GWG Score: 1* Exceptional merit, warrants funding

- Votes for score of 1: **8**
- Votes for score of 2: **6**
- Votes for score of 3: **1**

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: $4,268,865

*Revised application score - originally scored “2” (0-13-0)

CIRM
CALIFORNIA'S STEM CELL AGENCY

GWG Vote on Review Process

1. All members: “The review was scientifically rigorous, there was sufficient time for all viewpoints to be heard, and the scores reflect the recommendation of the GWG.”

2. ICOC patient advocate members: “The review was carried out in a fair manner and was free from undue bias.”

All members voted unanimously in favor of 1 (20-0)

ICOC members voted unanimously in favor of 2 (6-0)

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