CIRM
CALIFORNIA'S STEM CELL AGENCY

Funding Opportunity Concept Changes

Gil R Sambrano
Vice President of Portfolio Development and Review
California Institute for Regenerative Medicine

TRANSFORMING medicine lives futures

November 15, 2018

Add ”Gene Therapy” to CIRM Project Eligibility

- Gene therapy involving stem or progenitor cells is currently eligible.
- Propose to include gene therapy approaches that do not involve stem cells.
- Requires GWG 2/3 majority vote deeming projects “vital research opportunity”.

Add ”Gene Therapy” to CIRM Project Eligibility

A gene therapy approach:

- (i) that targets a stem cell for its therapeutic effect, OR any other somatic cell if deemed a “vital research opportunity” by the CIRM Grants Working Group; AND
- (ii) is intended to replace, regenerate, or repair the function of aged, diseased, damaged, or defective cells, tissues, and/or organs; AND
- (iii) is being developed for a rare or unmet medical need unlikely to receive funding from other sources.

Add ”Gene Therapy” to CIRM Project Eligibility

CIRM considers gene therapy to mean a human therapeutic intervention intended to:

1) alter the genomic sequence of cells or

2) alter the cellular lineage via gene delivery (i.e., direct lineage reprogramming).

The intervention may include strategies to repair a disease-causing gene sequence, remove or inactivate a disease-causing gene, or introduce new or modified genes that augment the therapeutic potential of the target cells.

Process for Establishing a "Vital Research Opportunity"

1. Applications that meet the gene therapy eligibility criteria will be accepted for GWG review.

2. The GWG will take a vote on whether each application in this category represents a "vital research opportunity" for CIRM.

3. If the GWG does not agree that the application is a "vital research opportunity" then the application will be withdrawn from further consideration.