CIRM
CALIFORNIA'S STEM CELL AGENCY

Funding Opportunity Concept Changes

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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 Eligibility

1. Convene GWG to consider concept of gene therapy as proposed.

2. Take GWG vote on whether these efforts represent a “vital research opportunity” for CIRM.

3. Assuming a positive vote, accept all applications that meet the new criteria without modifying the current scientific review process.