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CALIFORNIA'S STEM CELL AGENCY

Quest and Challenge Programs: GWG Recommendations

Gil R. Sambrano, Ph.D.

Director of Portfolio Development & Review
California Institute for Regenerative Medicine

PROGRAM OVERVIEW:

QUEST (DISC 2) RESEARCH PROJECTS

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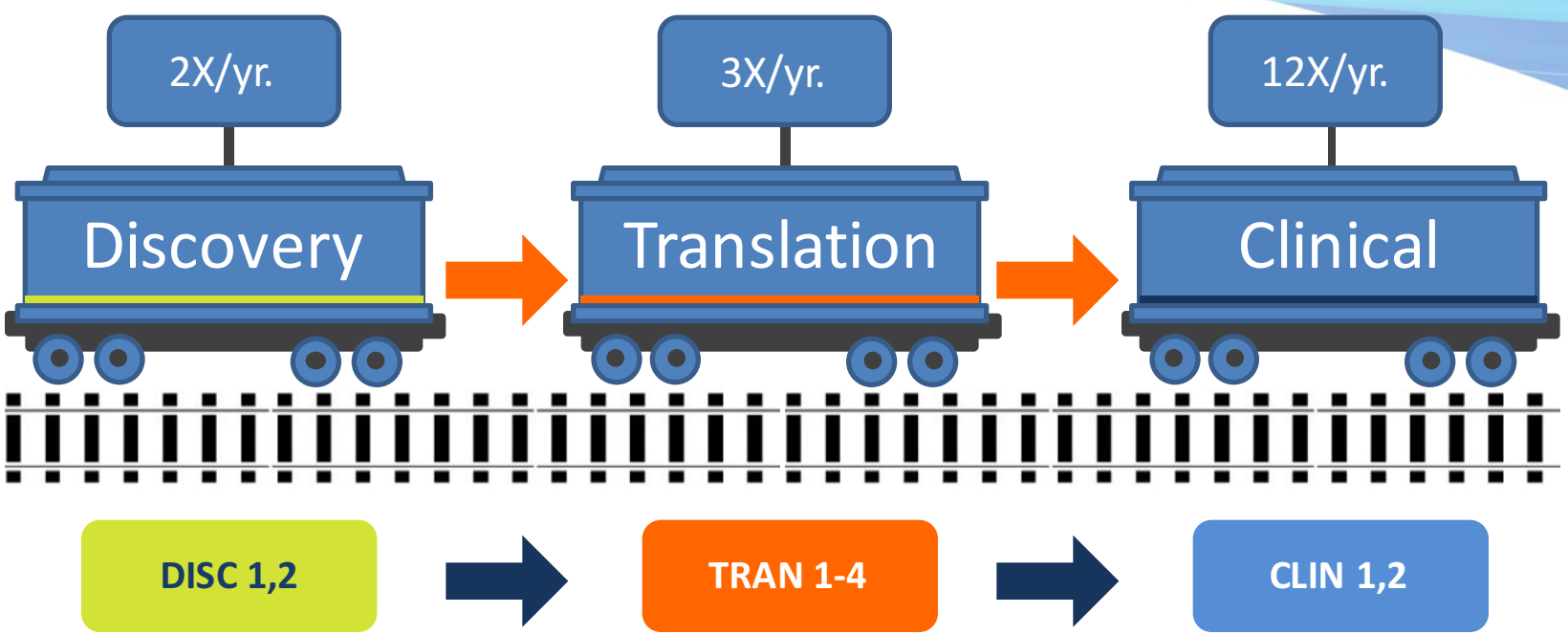
Where the Discovery Program Fits



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CIRM 2.0 Quest Program

Objective

The Quest Program promotes the discovery of promising new stem cell-based technologies that could be translated to enable broad use and ultimately, improve patient care.

Projects funded through the Quest Awards should propose technology that is uniquely enabled by human stem/progenitor cells or directly reprogrammed cells, or uniquely enabling for the advancement of stem cell-based therapies.

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Key Points

- We looked for projects with the capability to develop a novel candidate product (i.e., therapeutic, diagnostic, medical device, or tool) that would be ready for translational studies within two years.

- The product type (i.e., therapeutic, diagnostic, medical device, or tool) determined the specific outcomes expected and readiness for translational studies.

- If successfully realized, the candidate offered the potential to improve patient care **or** to facilitate the discovery, development or use of stem cell-based therapies.

Review Criteria

1. Does the project hold the necessary significance and potential for impact?
2. Is the rationale sound?
3. Is the project well planned and designed?
4. Is the project feasible?

RECOMMENDATIONS OF THE GWG FOR THE QUEST REVIEW

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Scoring System

- **Score of "85-100"**

  Recommended for funding, if funds are available

- **Score of "1-84"**

  Not recommended for funding

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

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DISC 2: GWG Recommendations

	DISC 2 Apps	Funds
Recommended for funding Score 85-100	5	$9,442,218
Not recommended for funding Score 1-84	38	$56,445,840

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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 (20-0)

Patient Advocate GWG members voted unanimously in favor of 2 (6-0)

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DISC 2: CIRM Team Recommendations

	DISC 2 Apps	Funds
Score 85-100 Recommended for funding	7*	$13,644,906
Score 1-84 Not recommended for funding	27**	$40,153,943

*CIRM Team Recommendations	Score	Median	# GWG who scored 85-100	# GWG who scored 1-84
DISC2-09073	83	85	10	4
DISC2-08982	81	85	10	4

** There are 9 withdrawn applications.

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PROGRAM OVERVIEW:

CHALLENGE (DISC 3.1) RESEARCH PROJECTS

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CIRM 2.0 Challenge Program

Objective

The Challenge Program (DISC 3.1) will enhance the value of CIRM's Human Induced Pluripotent Stem Cell (hiPSC) bank for disease modeling, target discovery, drug discovery, and development through the acquisition and addition of genetic data for up to 3000 disease-specific and control stem cell lines.

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Key Points

- We looked for the **one grantee** that would enhance the value of CIRM's Human Induced Pluripotent Stem Cell (hiPSC) bank for disease modeling, target discovery, drug discovery, and development though the acquisition and addition of genetic data for up to 3000 disease-specific and control stem cell lines.

- The overall project deliverable is a comprehensive genetic profile of CIRM's Human Induced Pluripotent Stem Cell Bank to serve as a catalyst for further use in disease associated allele discovery, drug screening, and drug safety assessment.

Review Criteria

1. Does the project hold the necessary significance and potential for impact?
2. Is the rationale sound?
3. Is the project well planned and designed?
4. Is the project feasible?

Scoring System

- **Score of "85-100"**

  Recommended for funding.

  Only the application with the highest average score will have the GWG's recommendation to fund.

- **Score of "1-84"**

  Not recommended for funding.

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

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DISC 3.1: GWG Recommendations

Application #	Score	Budget
DISC3.1-09167	**88**	**$2,000,000**
DISC3.1-09024	85	$1,999,998
DISC3.1-09175	81	$2,000,000
DISC3.1-09176	74	$2,737,608
DISC3.1-09177	68	$1,999,154

Green shading indicates the GWG's recommendation for funding.

The CIRM Team concurs with the GWG recommendation.

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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 (16-0)

Patient Advocate GWG members voted unanimously in favor of 2 (6-0)

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