

SOMETHING BETTER THAN HOPE

Grants Working Group Recommendations: TRAN Review

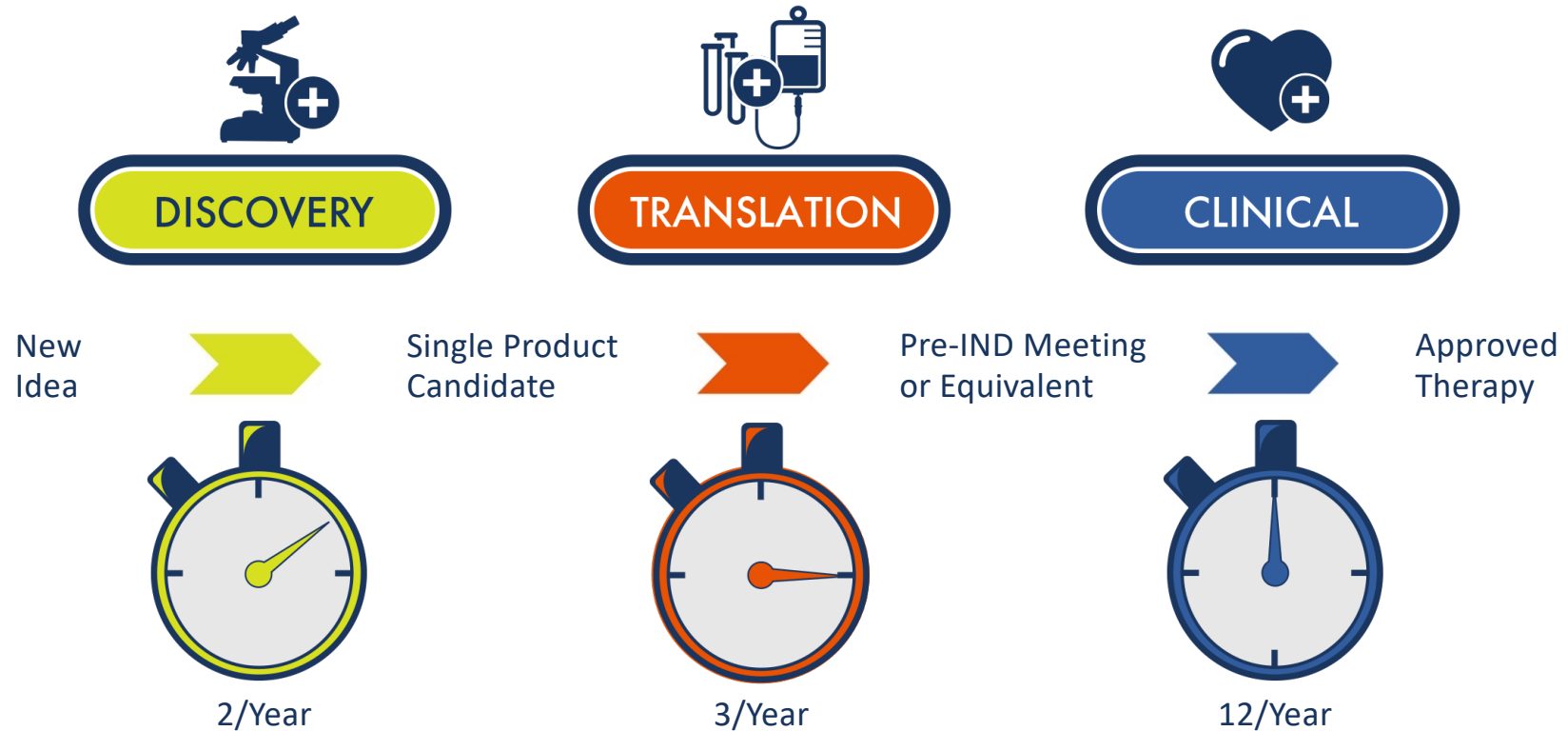
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CIRM
CALIFORNIA / STEM CELL AGENCY

CIRM Has Relunched Recurring Core Funding Opportunities for Therapy Development

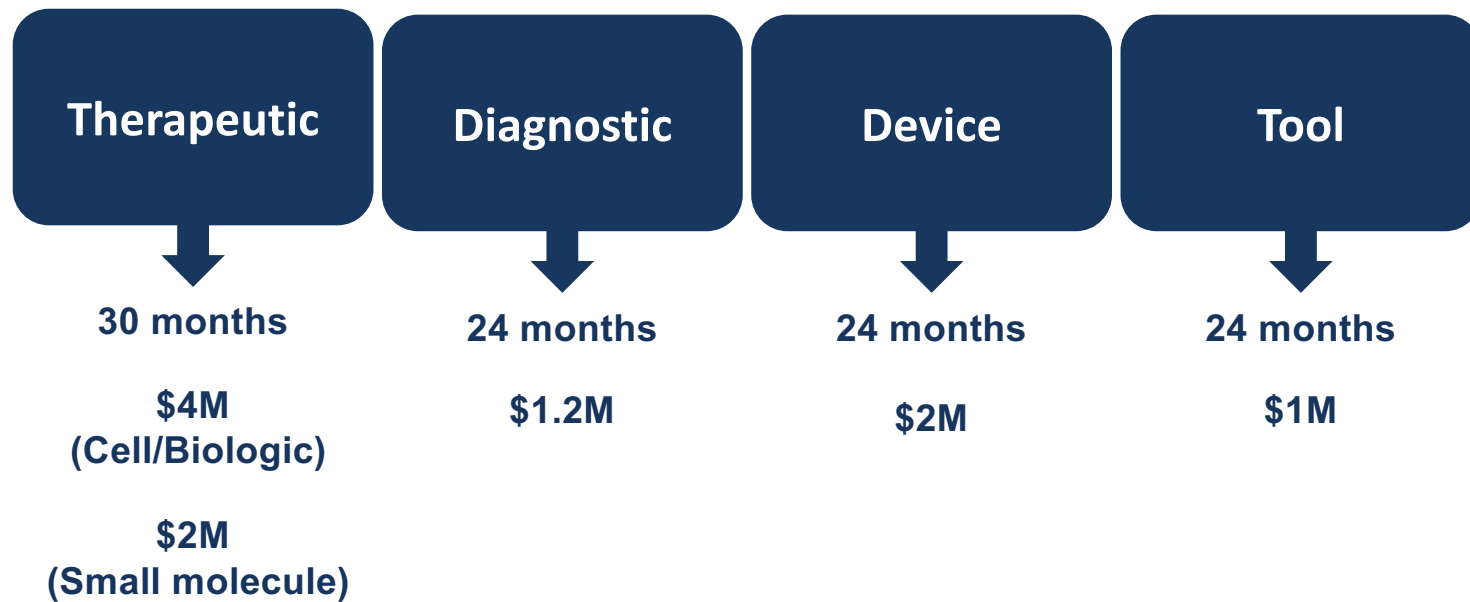


CIRM Translational Research Program (TRAN)

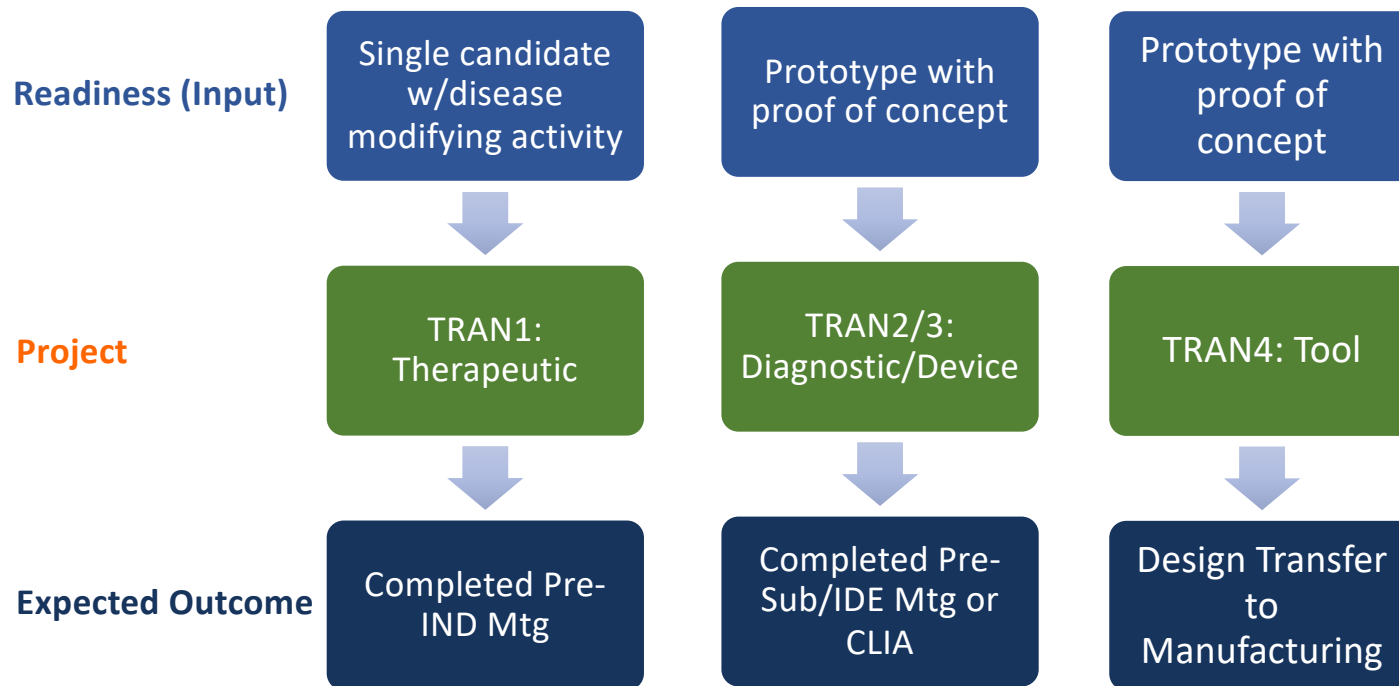
Objective

To support promising stem cell-based projects that accelerate completion of translational stage activities necessary for advancement to clinical study or broad end use.

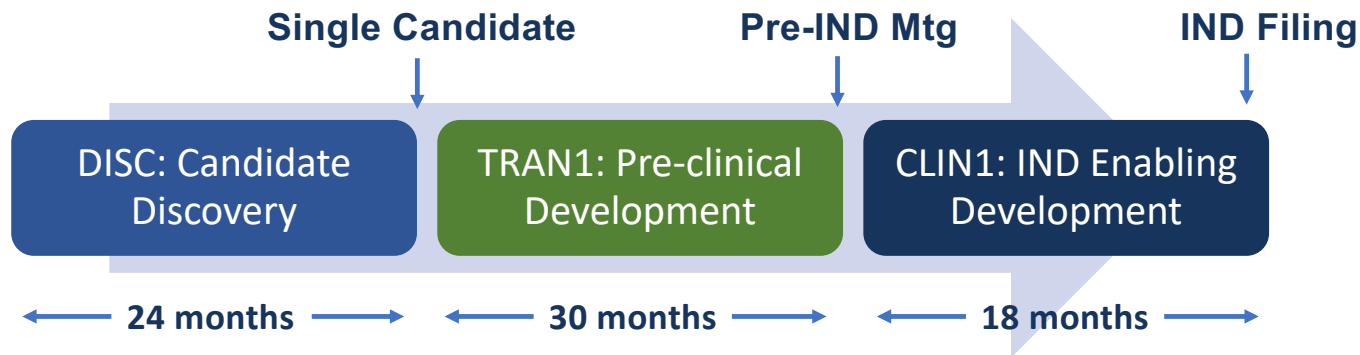
CIRM Translational Program (TRAN)



CIRM Translational Program (TRAN)



Accelerating Therapeutic Development



Expected **outcomes** of TRAN1 applications:

- Pre-IND meeting conducted and formal meeting minutes forwarded to CIRM
- Pivotal IND-enabling preclinical studies protocols drafted
- GMP-compliant Master Cell Bank and Working Cell Bank established
- Clinical indication defined and draft clinical protocol synopsis developed

Key Points for Reviewers

- The review criteria are the same for all 4 product types.
- The goal is to identify the projects that are most likely to achieve the objective of this program.
- There is no need to reserve or balance award slots of any product type at this review.
- Remember to account for differences in product type when evaluating activities and outcomes proposed.

Review Criteria

1. Does the project hold the necessary significance and potential for impact? (i.e., what value does it offer; is it worth doing?)
2. Is the rationale sound? (i.e., does it make sense?)
3. Is the project well planned and designed?
4. Is the project feasible? (i.e., can they do it?)
5. Does the project address the needs of the underserved?

Addressing Needs of the Underserved:

GWG 5th Review Criterion

5. Does the project serve the needs of underserved communities?

- Does the project plan and design adequately address and account for the influence of race, ethnicity, sex and gender diversity?
- Would the project outcomes inform the development of a product or tool that serves the unmet medical needs of the diverse California population, including underserved racial/ethnic communities?

Data Sharing Plan: New application element

- Sharing of data and knowledge produced from CIRM-funded projects is key to advancing the field of regenerative medicine and accelerating treatments to patients.
- CIRM requires its awardees to develop and execute a Data Sharing Plan that includes management and preservation of data and making applicable data available to the broader scientific community.

Instructions in Application:

Describe the proposed plan to make available data generated from the project. The description should include the type(s) of data expected to be produced, how the data will be managed, what data will be shared and how (i.e., repository), justification for not sharing certain data, timeline for data sharing and expected costs.

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.

The **median** of all individual GWG scores determines final score.

Minority Reports

- Under Prop 14, any application that is not recommended for funding by the GWG, but which had 35% or more members score to fund the application must include a minority report.
- The minority report is included in the review summary and provides a brief synopsis of the opinion of reviewers that scored the application 85 or above.

GWG Recommendations

	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	2	\$8,567,606	\$60,000,000
Not recommended for funding Score 1-84	11		

For each award, the final award amount shall not exceed the amount approved by the ICOC Application Review Subcommittee and may be reduced contingent on CIRM's assessment of allowable costs and activities.

Overview of Recommended Applications

TRAN1-12245

TITLE: Development of novel synNotch CART cell therapy in patients with recurrent EGFRvIII+ glioblastoma

DISEASE INDICATION: Glioblastoma

PRODUCT TYPE: Cell therapy

APPROACH: An autologous CAR T cell therapy that targets multiple antigens on glioblastoma tumor cells

TRAN1-12258

TITLE: CAR-Tnm cell therapy for melanoma targeting TYRP-1

DISEASE INDICATION: Melanoma

PRODUCT TYPE: Cell therapy

APPROACH: An autologous CAR naïve/memory T cell therapy that targets a novel antigen TYRP-1 on melanoma tumor cells