

Real Life™

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Vice President, Portfolio Development and Review

Grants Working Group Recommendations TRAN

November 29, 2022

CIRM
CALIFORNIA'S STEM CELL AGENCY

OUR MISSION

Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world





DISCOVERY



TRANSLATION



CLINICAL

New
Idea



2/Year

Single Product
Candidate



2/Year

Pre-IND or
Equivalent



12/Year

Approved
Therapy

TRAN1
Therapeutic

30 months
\$4M
(Cell/Biologic)
\$2M (Drug)

TRAN2
Diagnostic

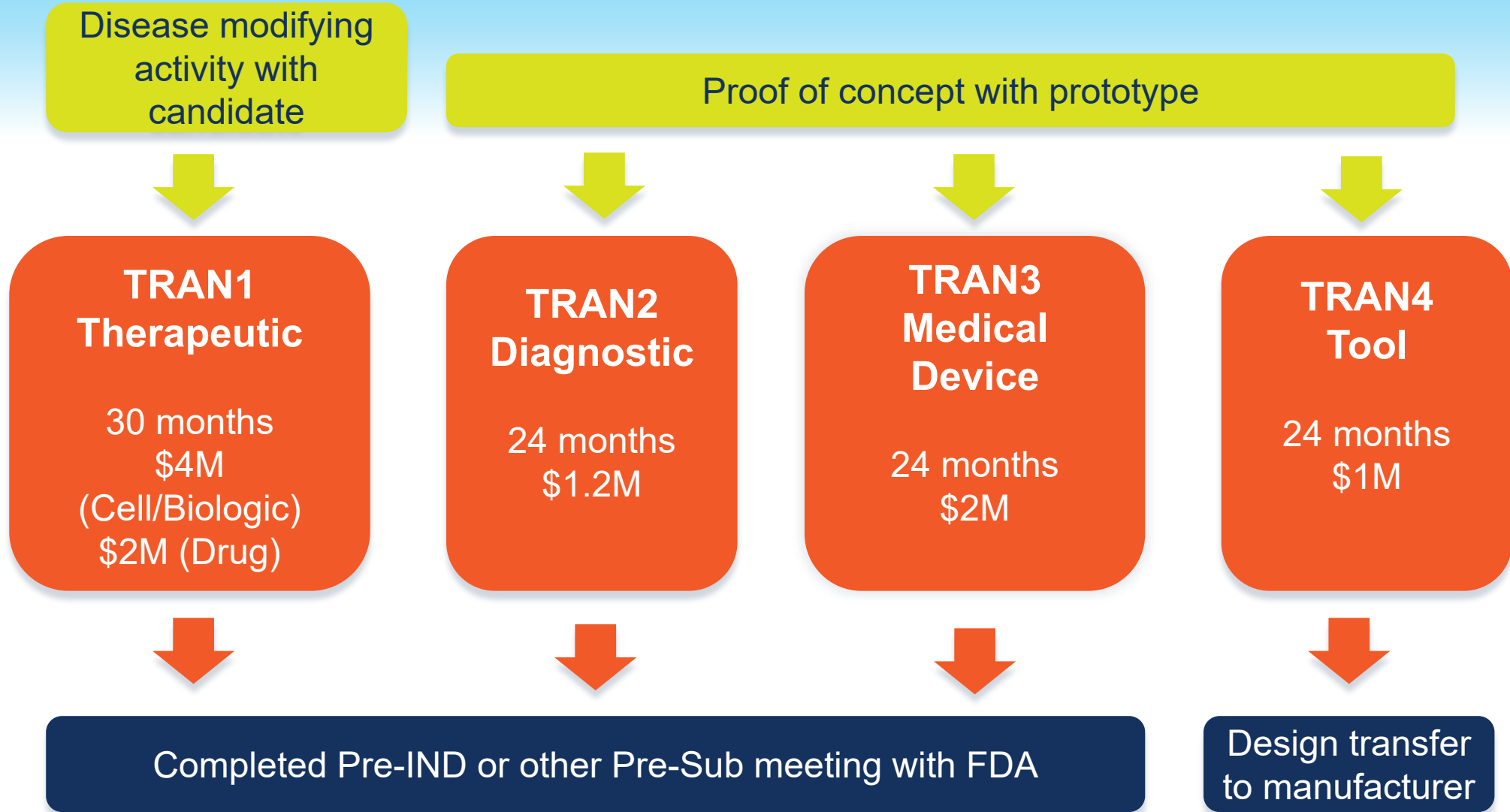
24 months
\$1.2M

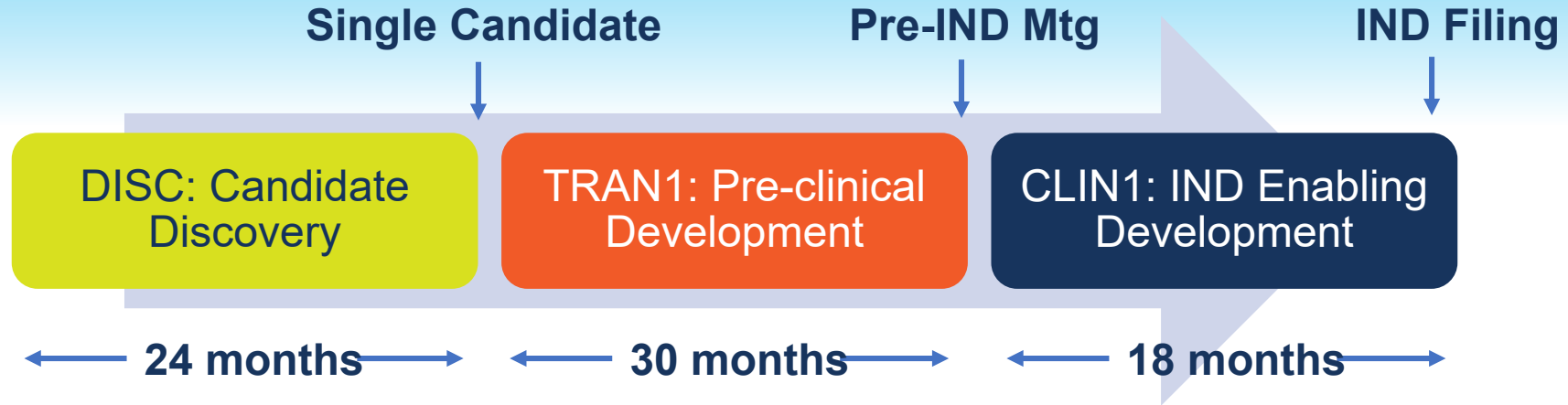
TRAN3
**Medical
Device**

24 months
\$2M

TRAN4
Tool

24 months
\$1M





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

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 underserved communities?

- **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.

Scientific GWG
Member



Scientific evaluation (disease area expert,
regulatory, CMC, product development)
Provides scientific score on all applications

Patient Advocate
or Nurse GWG
Member



Patient perspective on significance and potential
impact, oversight on process
Provides a DEI score

Scientific
Specialist
(non-voting)



Scientific evaluation (specialized expertise as
needed)
Provides initial but not final scientific score

Board members with Conflicts of Interest for TRAN applications

Ysabel Duron

Karol Watson

GWG Recommendations

	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	4	\$16,232,581	\$70,400,000
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.

- 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.

CIRM Team concurs with the GWG recommendations for funding.

In addition, the CIRM Team supports the minority position for application TRAN1-13996 and recommends funding of this application.

App Number	Title	Funds Requested	Score
TRAN1-13996	Overcoming resistance to standard CD19-targeted CAR T using a novel triple antigen targeted vector	\$4,168,679	83