



Agenda Item #8
Programmatic Tools

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TRANSFORMING

medicine
lives
futures

June 28, 2018

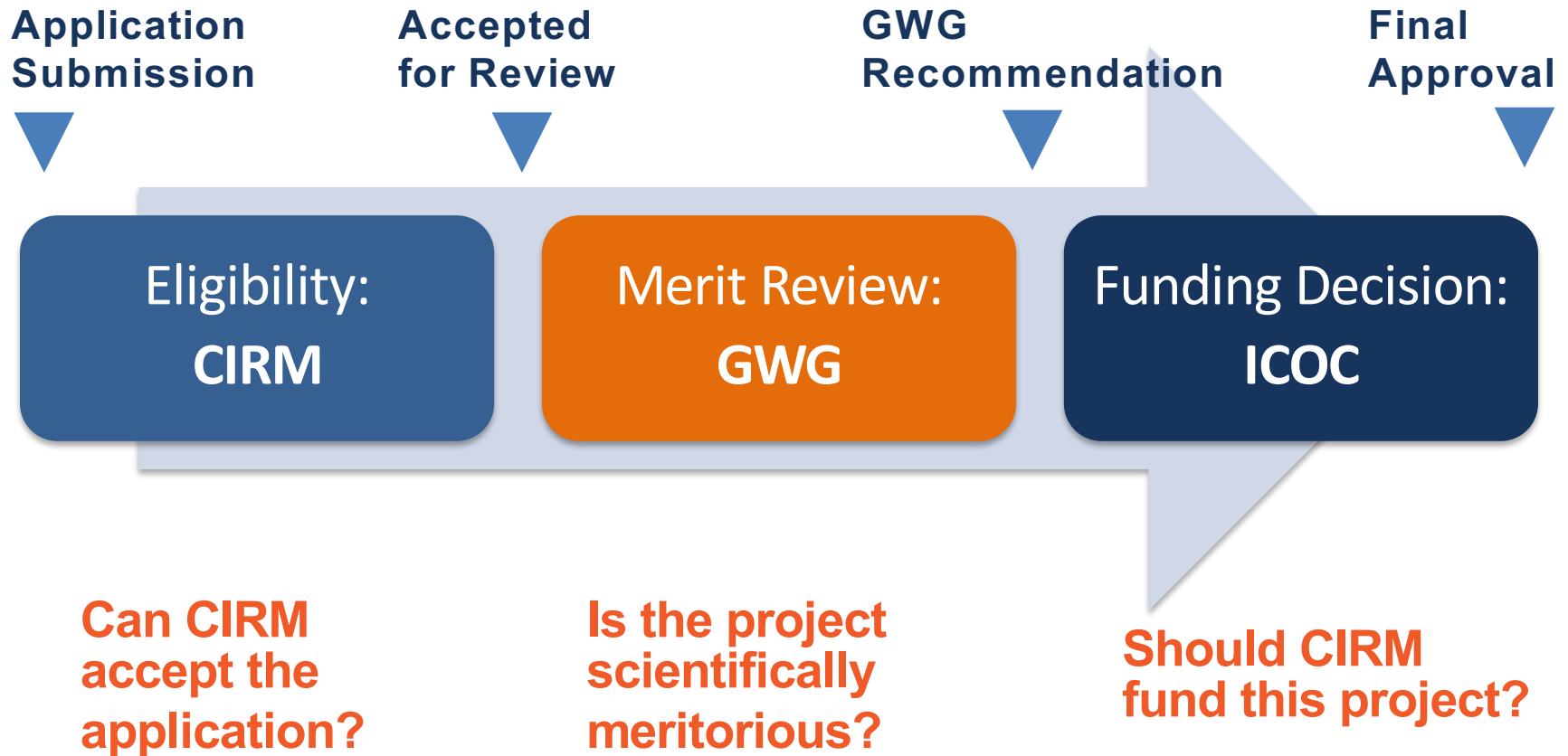


Presentation Outline

- Overview of Review Process
 - Gil Sambrano
- Discussion of Programmatic Review
 - Jeff Sheehy
- Possible Tools to Support Review
 - Gil Sambrano

Phases of the Review Process

Application Review Process



Merit Review:
GWG

Is the project scientifically meritorious?

- ✓ Does the project hold the necessary significance and potential for impact?

Includes value proposition, unmet medical need

- ✓ Is the rationale sound?

Scientific/clinical basis, supportive data

- ✓ Is the project well planned and designed?

- ✓ Is the project feasible?

Adequate resources, quality team, achievable timeline

Scoring for DISC and TRAN Applications

- **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.

Scoring for Clinical Applications

- **Score of “1”**

Exceptional merit and warrants funding.

- **Score of “2”**

Needs improvement and does not warrant funding at this time but could be resubmitted to address areas for improvement.

- **Score of “3”**

*Sufficiently flawed that it does not warrant funding and the same project should not be resubmitted **for at least 6 months**.*

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

Application Review Subcommittee

What is programmatic review?

- Subcommittee considers GWG and CIRM recommendations
- Subcommittee may consider additional factors:
 - CIRM mission
 - Portfolio make-up
 - RFA objectives
 - Unmet medical need
 - Budget

What other factors could or should be considered?

Possible Tools to Support ICOC Subcommittee Review

Potential Discussion Points

- **Annual Program Budget and Goals**
- **Value Proposition of Proposed Project**
 - *Patient population, competitive landscape*
- **Relevance of Project to Stem Cells**
- **Contribution to CIRM Portfolio**
 - *Disease area, current award overlap*
- **Previous CIRM Support of Project**

2018 Clinical Budget Status

End of June

Annual Allocation: \$130 million

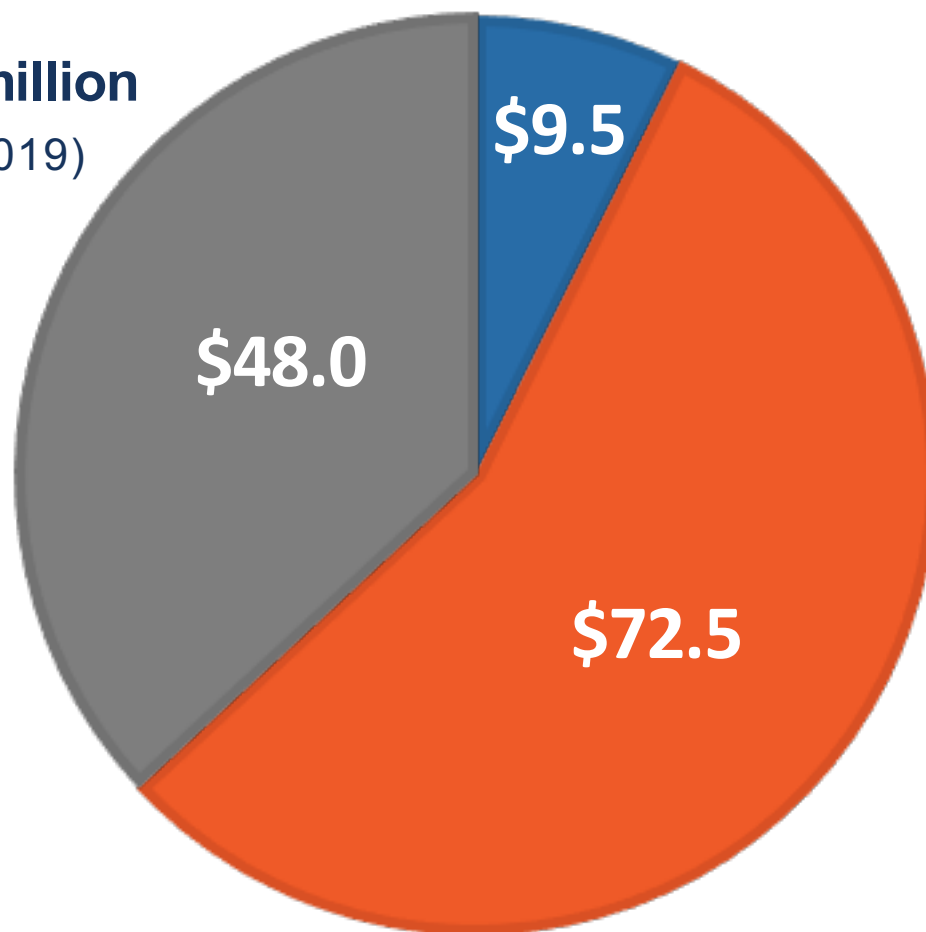
(Additional \$130 planned for 2019)

■ Amount Requested Today

■ Approved Awards

■ Unused Balance

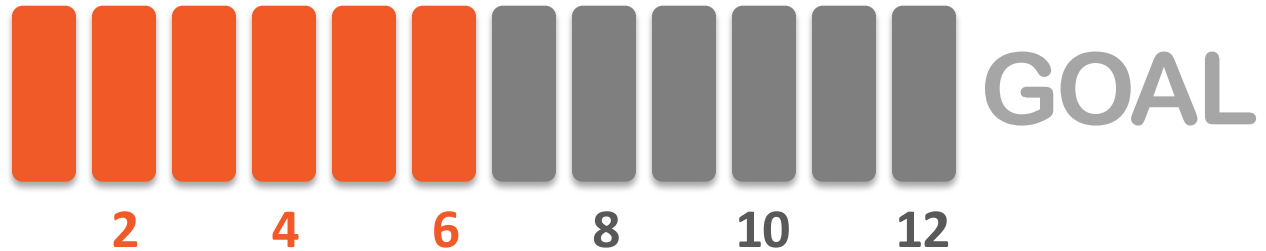
Amounts are shown in millions



2018 Clinical Award Targets

CLIN2

Clinical Trials



CLIN1

Late Stage
Preclinical



 Approved Award

 Awaiting Today's Approval

CLIN2-Example: Phase 1 Clinical Trial of Therapy for (X) Cancer

Project Summary

Therapy	Gene-modified dendritic cells
Indication	Adult patients with advanced (x) cancer
Goal	Complete a phase 1 clinical trial to assess safety, maximum tolerated dose, and response rate of therapy
Funds Requested	\$10,000,000 (\$400,000 Co-funding)

Maximum funds allowable for this category: \$12,000,000

CLIN2-Example: Phase 1 Clinical Trial of Therapy for (X) Cancer

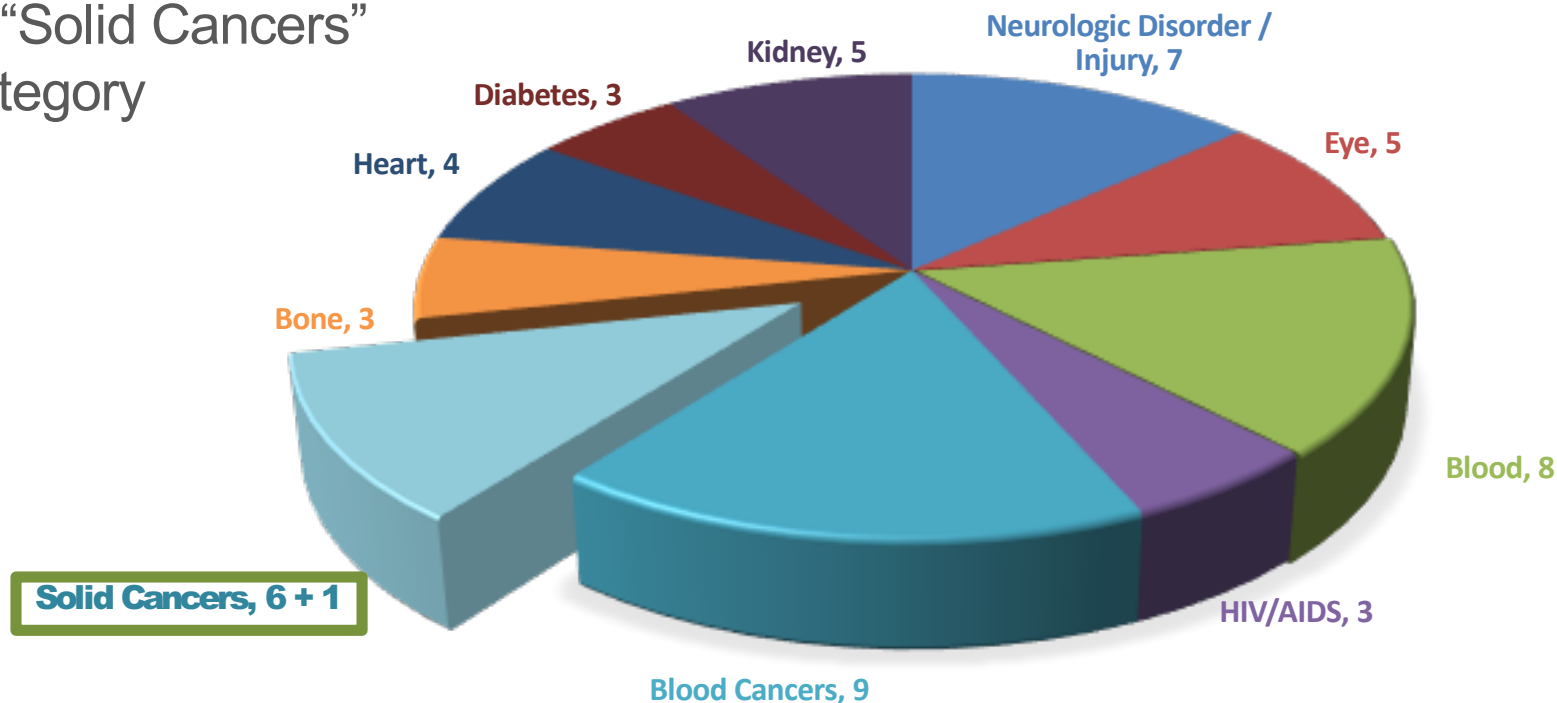
Potential impact: As stated by the applicant, about 350,000 people in the US are diagnosed with (x) cancer each year. About 75% of patients with (x) cancer will die of the disease within 5 years of diagnosis. If successful, the proposed therapy could impact a majority of (x) cancer patients.

Value Proposition: Statement that addresses competitive advantage, advancement over current standard of care, and overall value of proposed therapy .

Why a stem cell project: This is a cell therapy where monocytes are used to manufacture the dendritic cell product. Monocytes are capable of differentiation and retain an ability to divide.

Where does this project (CLIN2-XXXXX) fit into our clinical portfolio?

Adds a clinical trial to “Solid Cancers” category



Related CIRM Portfolio Projects

Application /Award	Project Stage	Project End Date	Indication	Candidate	Mechanism of Action
Current Application	Phase 1	N/A	Advanced (X) Cancer	Gene modified dendritic cells	Enhanced T cell activation
CLIN2-09577	Phase 1b/2	12/31/21	Advanced Solid Tumors	Anti Cd-47 and cetuximab antibodies	Phagocytosis of cancer stem cells
CLIN1-10893	IND	06/30/19	Solid Tumors	iPSC derived Natural Killer Cells	NK cell mediated elimination of cancer cells

Previous CIRM Funding for Project

Project Stage	Project Outcome	Project End Date
Translational	Conducted Pre-IND Meeting	01/01/2016
IND-Enabling	IND Filed	01/01/2018

{CIRM funds have not been previously awarded to this team or to earlier stages of this project.}

CLIN2-Example: Phase 1 Clinical Trial of Therapy for (X) Cancer

GWG Recommendation: Exceptional merit and warrants funding

Score	GWG Votes
1	15
2	0
3	0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$10,000,000*

*Final award shall not exceed this amount and may be reduced contingent on CIRM's final assessment of allowable costs and activities.

CLIN2-Example: Phase 1 Clinical Trial of Therapy for (X) Cancer

GWG Recommendation: Exceptional merit and warrants funding

Score	GWG Votes
1	8
2	6
3	0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: \$10,000,000*

*Final award shall not exceed this amount and may be reduced contingent on CIRM's final assessment of allowable costs and activities.

CLINICAL TRIAL APPLICATIONS

App#	Indication	Therapy	Project Stage	GWG Score [Votes]	Amount Requested
CLIN2-example	Sickle cell disease	Cell therapy (HSC)	Ph 1	1 [7-5-0]	\$5,000,000
CLIN2-example1	(X) cancer	Cell therapy	Ph 1	1 [15-0-0]	\$10,000,000
CLIN2-example2	Osteoarthritis	Small molecule drug	Ph 1	1 [8-6-0]	\$8,000,000

LATE STAGE PRECLINICAL APPLICATIONS

App#	Indication	Therapy	Project Stage	GWG Score [Votes]	Amount Requested
CLIN1-xxxyyy	Colon cancer	Cell therapy (MSC)	IND	1 [12-2-1]	\$4,000,000
CLIN1-aaabbc		Cell therapy (T cells)	IND	1 [8-5-2]	\$3,500,000