



SOMETHING
BETTER
THAN HOPE

Right now.

Grants Working Group

September 26, 2019

California Institute for Regenerative Medicine

CIRM
CALIFORNIA / STEM CELL AGENCY

Every Moment Counts. **Don't Stop Now.**

Agenda

- Welcome and Overview
- Breakout Group Discussions
- Lunch
- Presentation of Group Recommendations
- Final Recommendations
- Recognition of GWG Contributions to CIRM
- Reception

Our Mission
Accelerate Stem Cell Treatments
To Patients with
Unmet medical needs.



2004

CIRM created by Patient Advocates and California Stakeholders-Proposition 71

\$3B

Committed to CIRM Mission

1000

Cutting Edge Research & Transformative Programs funded

56 CLINICAL TRIALS

First in human, cell & gene medicine, some ready for final marketing approval

>1200 PATIENTS

Patients enrolled in Alpha Clinics Clinical Trials

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GWG Contribution to CIRM: 2005-2019

117 GWG REVIEWS

200+ AVERAGE APPS
SUBMITTED PER YEAR

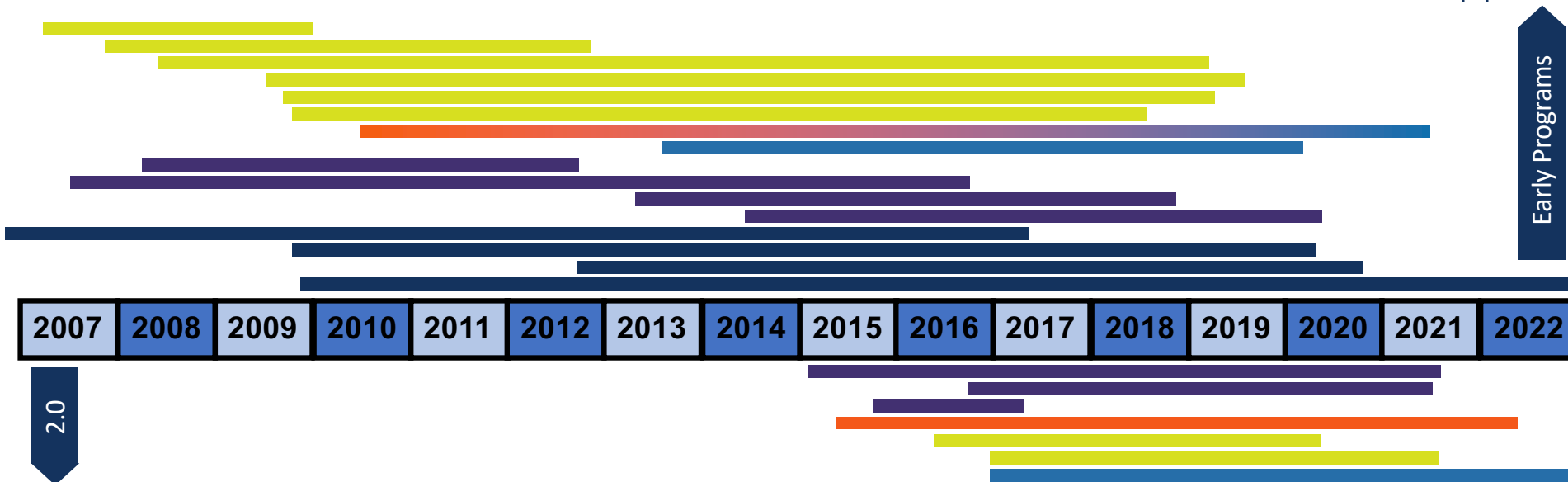
3000+ APPS REVIEWED

750+ TIER 1 SCORES

CIRM Programs Funded* (Years Active)

Early Programs:

- Seeded the field
- Launched the pipeline



Early Programs

Every Moment Counts. Don't Stop Now.

CIRM 2.0:

- Focused on therapeutic development
- Implemented operational improvements
- Streamlined meaningful goals into a 5 year strategy
- Formalized assessment of progress

*Each bar represents one major program



CIRM Investments: Five Funding Pillars



DISCOVERY



TRANSLATION



CLINICAL



INFRASTRUCTURE



EDUCATION

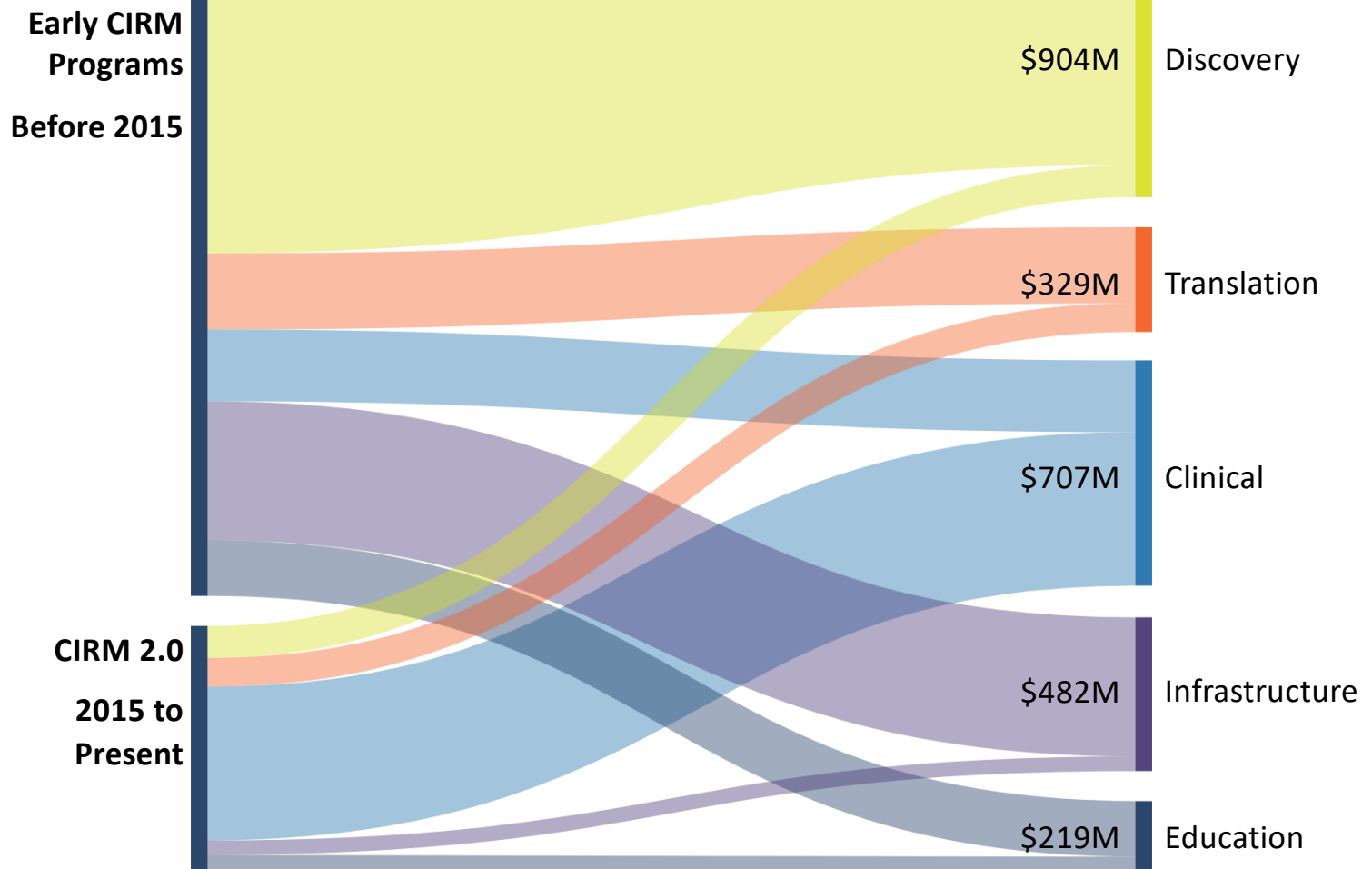
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CIRM Investments Across 5 Funding Pillars

\$2.6B
1000+
Awards





A self-assessment and realignment in 2014-15

- Operational improvements
- Strategy for next 5 years
- Setting ambitious and meaningful goals
- Tracking and measuring our progress

50
DISCOVER
NEW
CANDIDATES
INTO DEVELOPMENT

INCREASE
PROGRESSION
EVENTS

**50%
MORE**
ADVANCE

REFINE
ENACT
NEW REGULATORY
PARADIGM

**50%
LESS**
ACCELERATE
REDUCE
TRANSLATION TIME

50
VALIDATE
NEW
CLINICAL
TRIALS

50%
PARTNER
CLINICAL
PROGRAMS
WITH COMMERCIAL
PARTNERS

CIRM 2.0

CALIFORNIA'S STEM CELL AGENCY

2020 VISION

**SOMETHING
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CIRM 2.0

RAPID



Monthly Submission Cycle

Application to Award Start:
130 days

SEAMLESS

Candidate Discovery

Up to \$900K Direct

----Single Candidate----

Translational

Up to \$4M Direct

----Pre-IND Meeting----

IND Enabling

Up to \$6M

----IND----

Clinical Trials

Up to \$15M

COLLABORATIVE

CIRM

ALPHA STEM CELL CLINICS NETWORK

UC DAVIS
HEALTH

UCSF

City of
Hope

Alpha Clinic

UCLA UCI
Alpha Stem Cell Clinic

UC San Diego Health

Cell And Gene
Therapy Center

IQVIA™

CIRM
CALIFORNIA'S STEM CELL AGENCY

Bond Initiative

- A bond initiative to renew CIRM has been proposed for the November 2020 election
- Proposed initiative is expected to request \$5.5 billion
- CIRM is not involved in the initiative process, but if you have questions contact Americans for Cures (www.americansforcures.org)
- If initiative fails, CIRM will begin a wind-down process
- If initiative succeeds, CIRM will have a re-infusion of funds to continue existing programs and begin new ones

Meeting Goals

What should CIRM be thinking about now to prepare for a possible life beyond 2020?

- How can CIRM deliver the greatest impact in the future?
- What opportunities might CIRM seize to accelerate the field?
- What challenges must be addressed?
- What types of CIRM programs should be sustained or expanded?
- What is missing, or needs more support?

Goals for Morning Breakouts

Consider central questions and propose 3 or more recommendations to CIRM.

- Basic and Discovery Research
- Translational Research
- Clinical Research
- Training and Education
- Infrastructure Programs

Afternoon Group Share-out Format

Share ~3 initial recommendations to CIRM (5 min)

Discuss feedback, questions to the group, pain points (20 min)

Conclusions/summary (5 min)

- Basic and Discovery Research
- Translational Research
- Clinical Trials
- Training and Education
- Infrastructure Programs