Real Life™

June 2022 ICOC Meeting

President's Report

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President & CEO
June 27, 2022

CIRM
CALIFORNIA'S STEM CELL AGENCY

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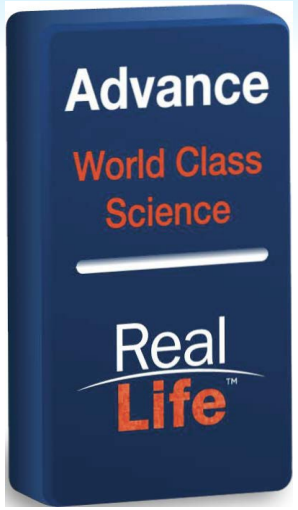
Real Life™

OUR MISSION

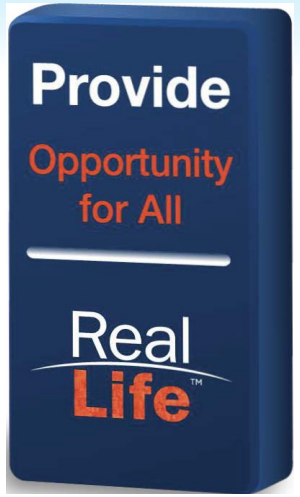
Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world

5-Year Strategic Plan Launch



- Develop shared resources
- Build knowledge networks

- Advance therapies to marketing approval
- Create a manufacturing partnership network
- Expand Alpha Clinics Network
- Create Community Care Centers of Excellence



- Build a diverse and highly skilled workforce
- Deliver a roadmap for access and affordability

2Q' 2022 Activities to Advance Prop 14 Strategic Goals

- ✓ Launched **DISC-0** to fuel basic and foundational research
- ✓ Launched the **data sharing and management plan guidelines** for CIRM awards
- ✓ ICOC Approved **19 DISC, 4 TRAN and 3 CLIN** awards
- ✓ Supported **3 conference awards** to promote knowledge sharing
- ✓ Conducted the **PSC Webinar** to exchange knowledge on selection of hPSC lines
- ❑ **2022-2023 Research Budget for Board Consideration today**

- ✓ Joined **the Bespoke Gene Therapy Consortium**
- ✓ Received applications for **Alpha Clinics Network Expansion**

- ❑ Patient support concept under development – **informed by AAWG Meetings (May 17 & June 21)**
- ✓ Received applications for **new EDUC5-COMPASS program** targeting a unique niche of underserved and underrepresented student communities

CIRM Funding Pillars

Q'2 2022 Awards

EDUCATION

3 Conf Awards

- 2022 CIRM SPARK Conference
- International Society for Stem Cell Research (ISSCR)
- 22nd Annual UC Symposium on Bioengineering & Biotechnology Industry

DISCOVERY

19 DISC Awards

- Neuro, blood diseases, heart, cancer, and more

TRANSLATION

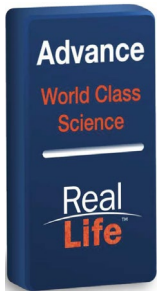
4 TRAN Awards

- Eye, heart, cancer, and kidney
- 2 gene therapy, 1 gene-modified cell therapy, and 1 device

CLINICAL

3 CLIN Awards
80 Clinical Trials Date

- Cancer, dysphagia, and epilepsy
- All cell therapy approaches



Stem Cell and Regenerative Medicine Conferences

American Society of Gene + Cell Therapy (ASGCT 2022)

- **3** talks by **CIRM grantees:** Don Kohn **(Outstanding Achievement Award)**, Stephanie Cherqui, Mark Tuszynski

International Society for Cell Therapy (ISCT 2022)

- **6** plenary presentations and roundtables

  CIRM grantees: Morton Cowan, Howard Foyt, Maria Grazia Rancarolo, David Miklos, Mark Walters, and Fyodor Urnov
- **Presentation on CIRM Programs-** Shyam Patel, Director of CIRM BD & Alliance Management
- **Regulatory Roundtable-** Abla Creasey, CIRM VP of Therapeutics Development
- **CIRM Booth -** CIRM team met with broad stakeholders

International Society for Stem Cell Research (ISSCR 2022)

- **32** talk and poster presentations

  CIRM grantees: Clive Svendsen, Kyle Loh, Cory Nicholas, Don Kohn, Yanhong Shi, Deepak Srivastava, and April Pyle

  14 CIRM Bridges Interns: poster presentations
- **Ethics Session on Public Engagement-** Kevin McCormack, CIRM, Director Patient Outreach, panelist
- **Public Symposium -** Jonathan Thomas CIRM ICOC Chair, Deepak Srivastava , Tom Carmichael and Adrienne Shapiro (Pt. Advocate)
- **DEI Workshop -** CIRM Team participation
- **Discussion on Equity, Diversity, and Inclusion -** Maria Bonneville, CIRM VP, discussant
- **Quality Standards special session-** Uta Grieshammer and Ross Okamura, CIRM Science Officers, discussants
- **CIRM Booth** – CIRM Team met with broad stakeholders

Partnerships to Advance Science and Accelerate Therapies

- **CIRM joined the Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium (AMP® BGTC)**
- Consortium of the NIH, FDA and over 20 private sector partners, managed by FNIH
- Goal is to create a standard blueprint for accelerated development of AAV gene therapies for rare genetic diseases
- Eligible CIRM funded programs will be part of this Consortium
- CIRM sits on the Steering Committee

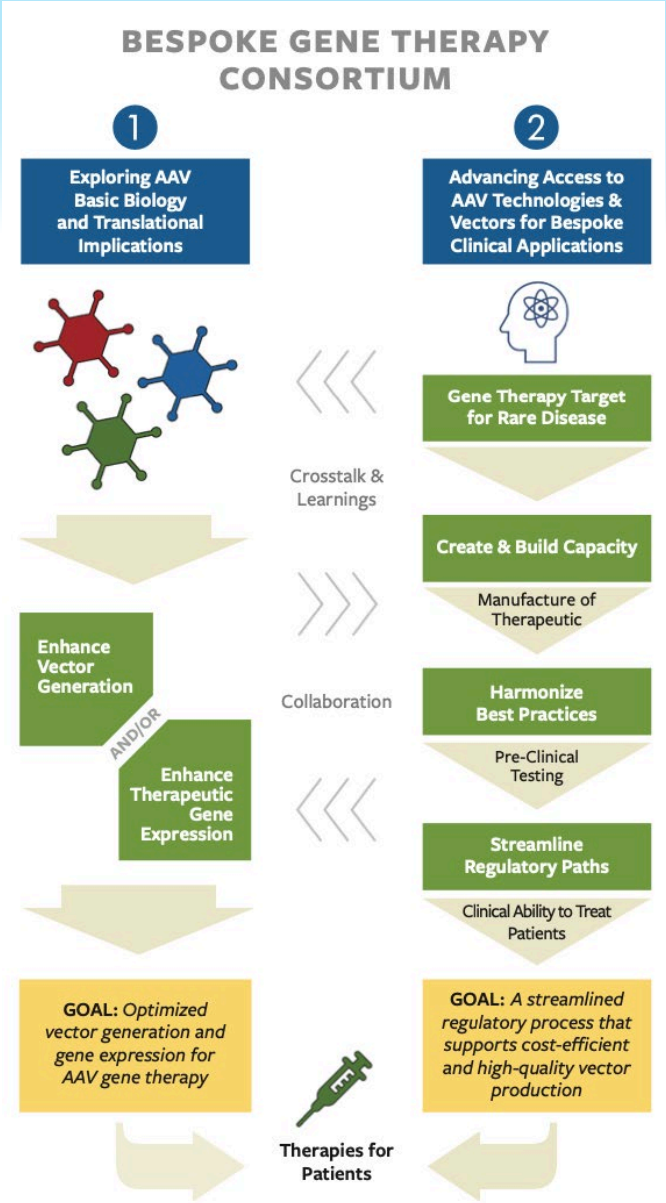


Figure from FNIH

Broad Stakeholder Engagement & Knowledge Sharing

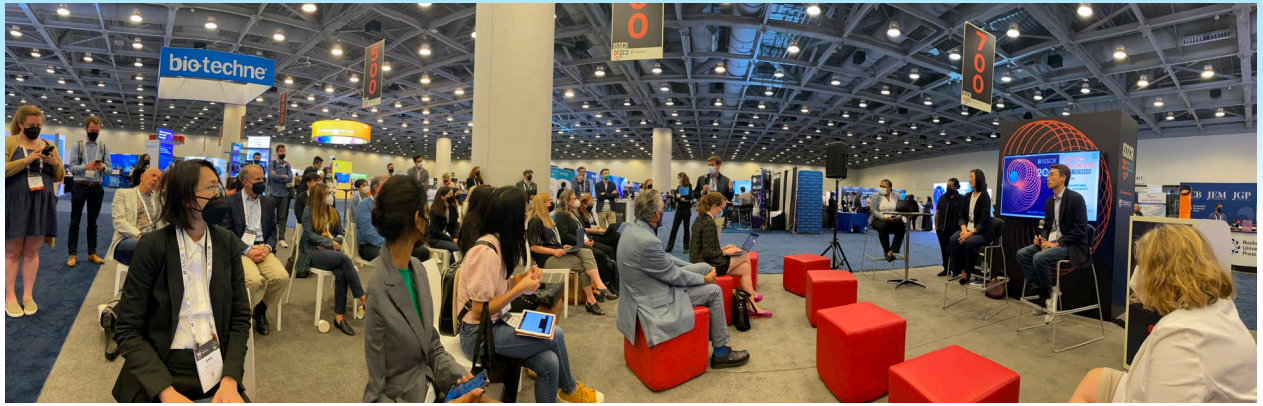


Photo provided b yWorld Medical Innovation Forum (Boston)