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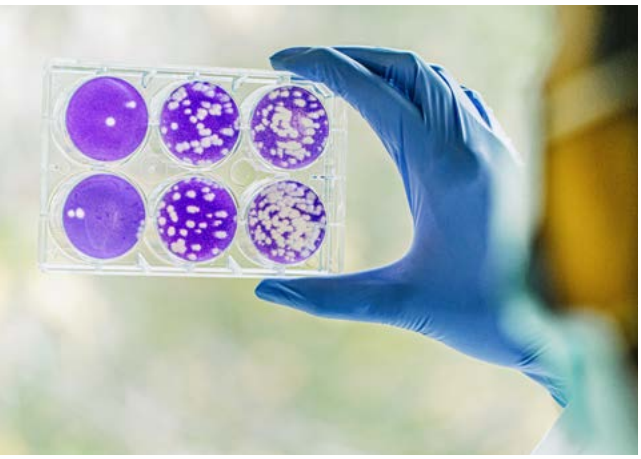

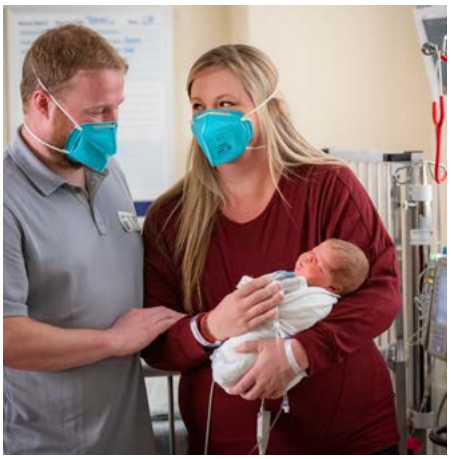

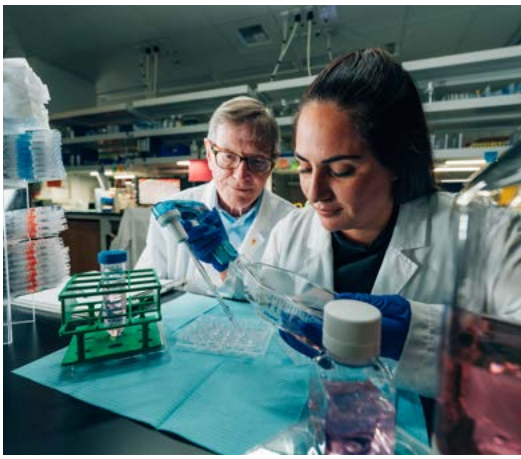

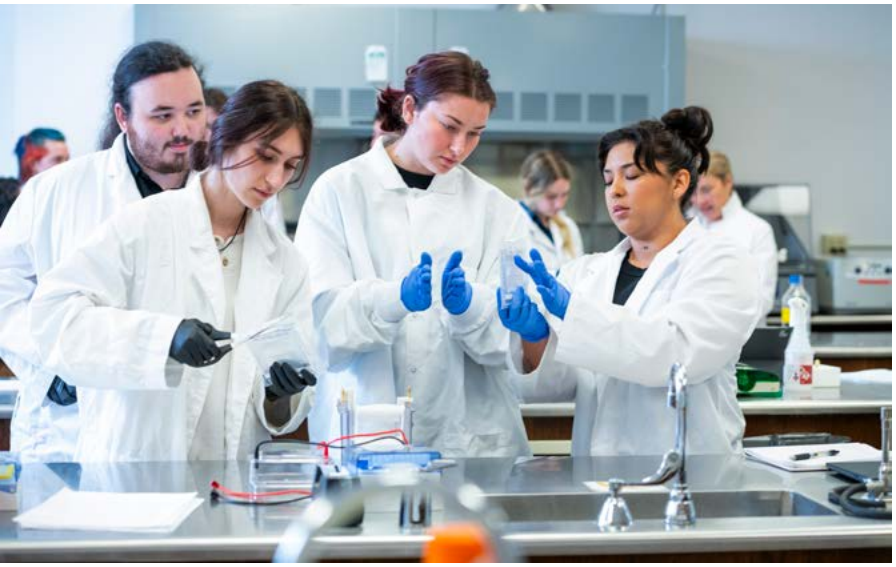

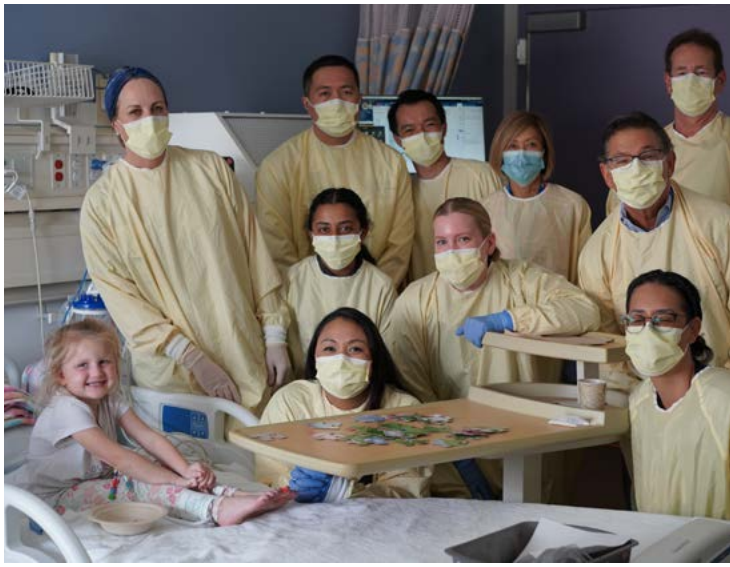

Strategic Allocation Framework (SAF) Recommendations

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Executive Summary

The California Institute for Regenerative Medicine (CIRM) has established itself as a leader in propelling stem cell research and regenerative medicine, dedicated to accelerating scientific discoveries into transformative treatments. However, with the rapid evolution of the field, CIRM is presented with the pivotal challenge of strategically allocating its remaining $3.86 billion across a spectrum of initiatives to optimize impact and further propel the field.

To enhance the efficacy of its funding strategy, CIRM has developed the Strategic Allocation Framework (SAF)—a structured and data-driven approach to prioritize resource allocation. This framework emerged from a critical need to balance increasing demands with finite resources while maintaining California's leadership in regenerative medicine. The goal is to maximize CIRM's impact, focusing on areas where it can significantly advance the field of regenerative medicine.

Regenerative Medicine Landscape

The landscape of regenerative medicine is marked by an accelerating pace of scientific breakthroughs, the emergence of innovative therapeutic products, and an expanding array of clinical trials targeting a wide spectrum of diseases and injuries. This dynamic environment presents both opportunities and challenges for CIRM. While the field holds immense promise, CIRM's resources are finite, and the demand for funding continues to grow at an unprecedented rate.

The SAF Process

The SAF process at CIRM was an iterative, data-driven approach to ensure the alignment of initiatives with CIRM's overall mission. This involved defining high-level guiding questions, gathering relevant data, and conducting thorough analyses to draw insights and develop a well-rounded understanding of the potential outcomes of each recommendation. This collaborative effort spanned CIRM teams, external consultants, and community inputs.

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Impact Goals

At the outset of the strategic planning process, CIRM began with a working hypothesis built around four key categories that drive its overarching mission. This initial hypothesis formed the basis for developing a comprehensive set of impact goals.

The result was a set of six final recommended impact goals (framed within the four categories) that will guide our actions and funding priorities. These goals are designed to accelerate the discovery and translation of therapies, advance critical approvals for cell and gene therapies, improve accessibility and affordability, and ensure a diverse and skilled workforce capable of sustaining advancements in regenerative medicine.

CATEGORY 1—ACCELERATING DISCOVERY & TRANSLATION

- Catalyze the identification and validation of at least 4 novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California.
- Accelerate development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies.

CATEGORY 2—CELL & GENE THERAPY APPROVALS

- Advance 4-7 rare disease projects to BLA.
- Propel 15-20 therapies targeting diseases affecting Californians to late-stage trials.

CATEGORY 3—ACCESSIBILITY & AFFORDABILITY OF CIRM-FUNDED CELL & GENE THERAPIES

- Ensure that every BLA-ready program has a strategy for access and affordability.

CATEGORY 4—DIVERSE WORKFORCE DEVELOPMENT

- Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine.

The following sections will explore these impact goals in more detail, along with the specific recommendations developed to support their achievement.

Catalyze the identification and validation of at least 4 novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California.

RECOMMENDATIONS

Support comprehensive discovery research through DISC4 & DISC5 funding structures

- *Encourage collaborative, multidisciplinary innovation in stem cell and genetic research across diverse disciplines & disease indications with early engagement of industry to address reproducibility & scalability issues*

Establish a Data Coordinating and Management Center (DCMC) to streamline data management and enhance the utility of cross-disease data

- *Fund and develop a central hub for data coordination, facilitating better integration with consortia & research initiatives and enabling data science collaborative efforts via dedicated grant*

Accelerate development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies.

RECOMMENDATIONS

Pilot INFR Technology Platform Program to bridge the gap between research and commercialization

- *Foster partnerships between academic researchers & industry professionals to support multi-stakeholder technology incubation programs that achieve defined technology readiness levels thereby facilitating rapid application in cell & gene therapy development*

Advance 4-7 rare disease projects to Biologics License Application (BLA).

RECOMMENDATIONS

Accelerate Current rare disease therapy pipeline

- *Increase and scale CLIN4 funding to comprehensively address BLA readiness gaps in manufacturing clinical/ non-clinical research and pre-commercialization*

Pilot Platform-Based Therapy Development

- *Implement pilot platform-based approach for gene therapy development using life-threatening monogenic neurological disorders as a test case*

Propel 15-20 therapies targeting diseases affecting Californians to late-stage trials.

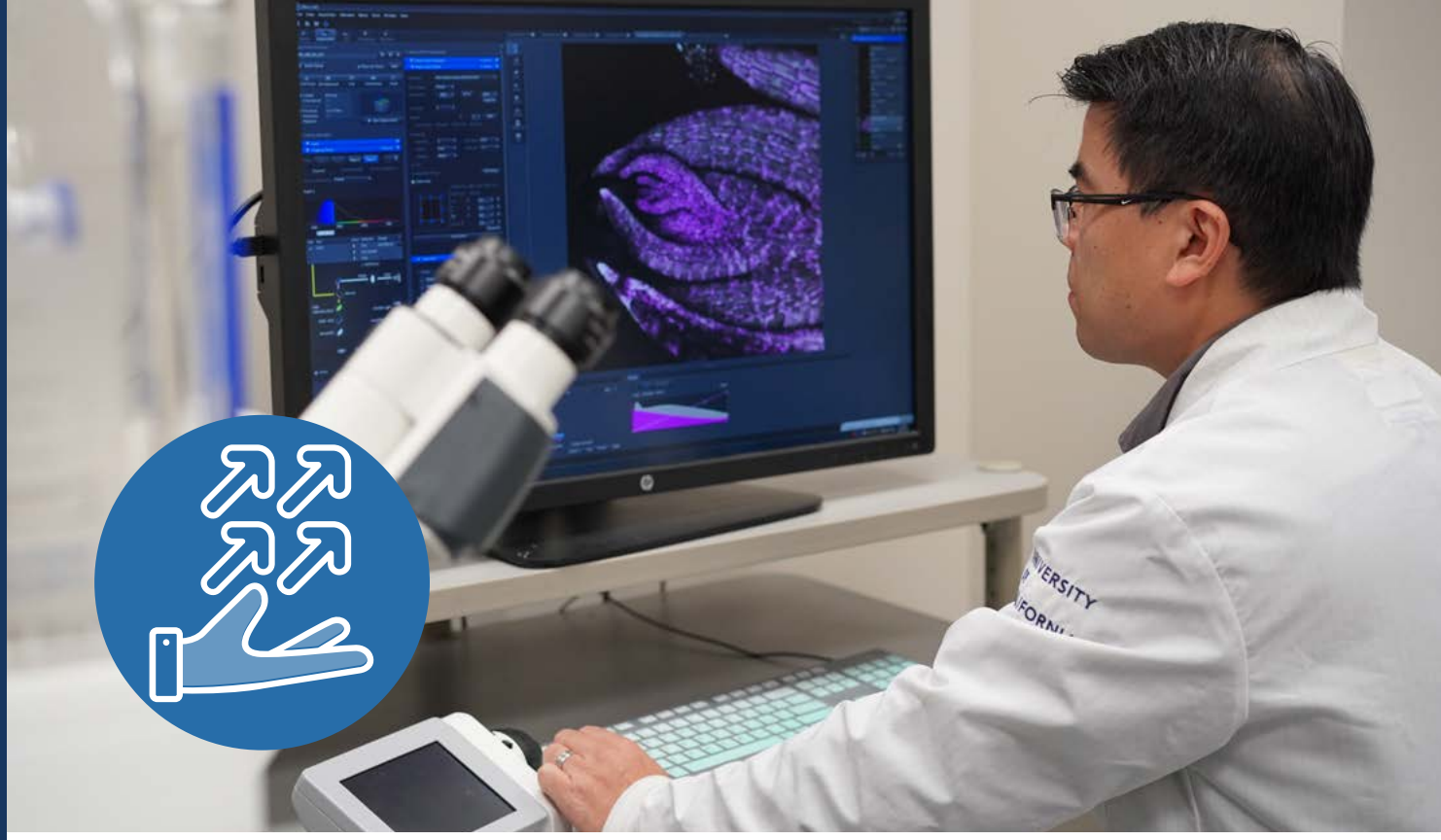
RECOMMENDATIONS

Streamline Preclinical Development Programs

- *Consolidate DISC2, TRAN 1-4, and CLIN1 to accelerate the preclinical development incentivizing multidisciplinary collaborations and rapid progression to IND. Incorporate prioritization of innovative therapies for diseases that affect Californians*

Update CLIN2

- *Allow for support of emerging novel clinical trial designs in CLIN2 program*
- *Incentivize stage-appropriate market access strategy development and pre-commercialization activities in the CLIN2 program*
- *Incorporate prioritization of innovative therapies for diseases that affect Californians*



Ensure that every BLA-ready program has a strategy for access and affordability.

RECOMMENDATIONS

Strengthen Clinical Infrastructure Connectivity

- *Build interconnectivity & performance metrics between CIRM Clinical Infrastructure (Alpha Clinics, CCCEs, PSP) to ensure enhanced referral enrollment & retention of California patients in clinical trials*

Support Development of Market Access and Reimbursement Strategies

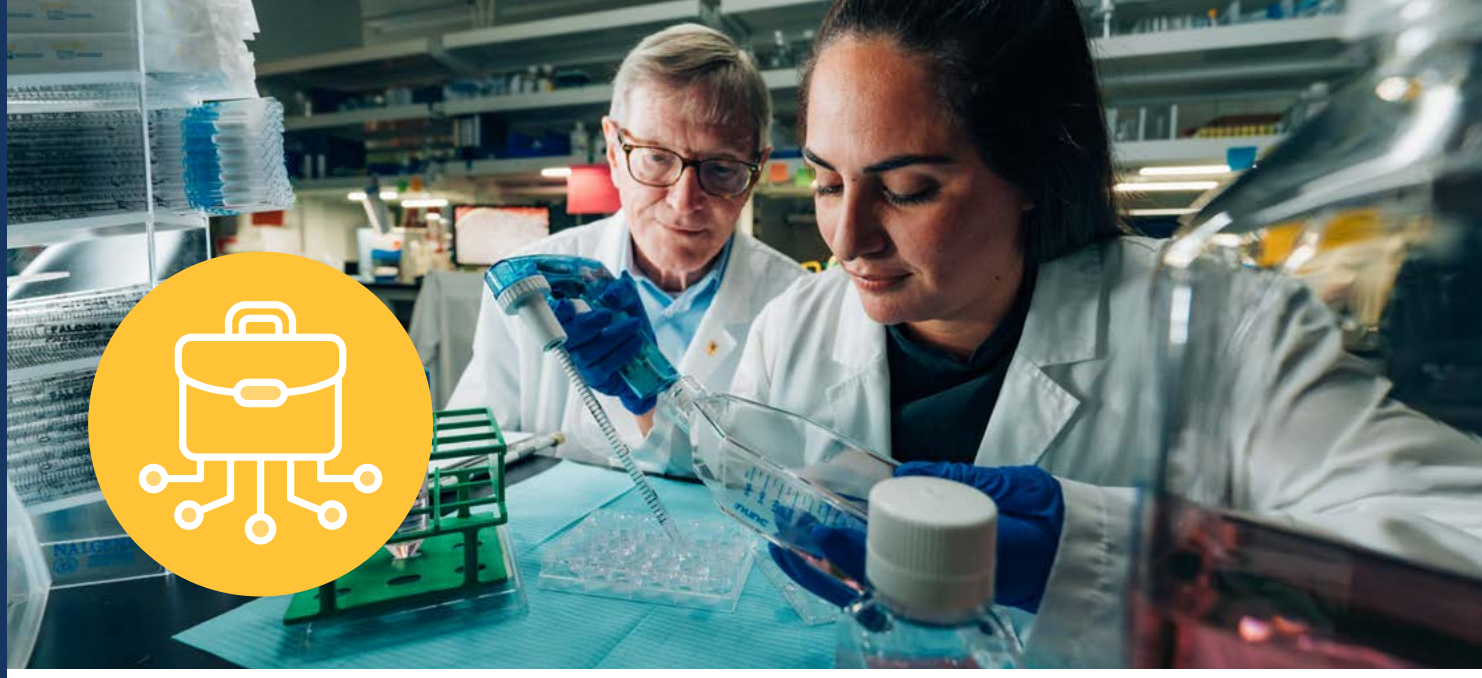
- *Resource clinical programs to support stage appropriate planning & evidence generation to inform robust market access & reimbursement strategies*

Influence Policy

- *Deploy AAWG resources to advocate for policies that advance access & reimbursement for regenerative medicines*

Enhance Partnerships

- *Engage state & national partners to align initiatives that expand sustainable access to regenerative medicines*



Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine.

RECOMMENDATIONS

Provide high-demand technical training via Bridges & COMPASS program updates

- *Increase training offerings diversify internship types & increase integration with CIRM R&D grants*

Create new EDUC program to develop hybrid skillsets

- *Implement new program structure to focus on cross-disciplinary internships*

Launch outreach campaigns to educate the public & increase diversity of California's regenerative medicine workforce

- *Develop programming to support outreach education efforts for K-12, teachers, & community members via collaboration with key stakeholders*

Restart Grantee Conference to Report SAF Goal Progress

- *Restart recurring grantee conference (timing TBD) with main objective of reporting progress on SAF goals*

Keep Conference Grants for Specific CIRM Needs (EDUC1 Mechanism 2)

- *Grantee retains the primary responsibility for planning directing and executing the proposed event. CIRM team will work closely with the grantee to design and implement an event responsive to a specific CIRM needs*