

Strategic Allocation Framework (SAF) Recommendations

The SAF was designed to focus CIRM's resources on measurable impact goals framed within four key categories: Accelerating Discovery & Translation, Cell & Gene Therapy Approvals, Accessibility & Affordability, and Diverse Workforce Development.

The following are goals and recommendations from the SAF process.

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.

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



ACCESSIBILITY & AFFORDABILITY OF CIRM-FUNDED CELL & GENE THERAPIES



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

DIVERSE WORKFORCE DEVELOPMENTS



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