Strategic Allocation Framework – Implementation Update

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CIRM
CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE

Presentation Overview

SAF Recap

Strategic Allocation Framework (SAF) – A structured and data-driven approach to prioritize resource allocation and provide recommendations to the ICOC for continued implementation of CIRM's strategic plan

Led to the following **Goals** and **Recommendations:**

Summary | SAF Goals & Recommendations (1/4)

Goals	Recommendations
1. Catalyze the identification and validation of at least 4 novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California	**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 & 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 grants
2. Accelerate development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies	**Pilot INFR Technology Platform Program to bridge the gap between research & 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

Summary | SAF Goals & Recommendations (2/4)

Goals	Recommendations
3. Advance 4-7 rare disease projects to BLA	**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
4. Propel 15-20 therapies targeting diseases affecting Californians to late-stage trials	**Streamline Preclinical Development Programs** Consolidate DISC2, TRAN1-4, 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 CLIN2 program Incorporate prioritization of innovative therapies for diseases that affect Californians

Summary | SAF Goals & Recommendations (3/4)

Goals	Recommendations
5. Ensure that every BLA-ready program has a strategy for access and affordability	**Strengthen Clinical Infrastructure Connectivity** Build interconnectivity & performance metrics between CIRM Clinical Infrastructure (Alpha Clinics, CCCEs, PSPs) 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

Summary | SAF Goals & Recommendations (4/4)

Goals	Recommendations
6. Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine	**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
Additional Recommendations	**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 need

Presentation Overview

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2   SAF Planning to SAF Implementation

3   Concept Development Timeline

4   Questions

SAF Planning

SAF Implementation

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Timeline | Concept Development & Amendments

Goal	Concept	Type
1	Revised DISC4/5 for Discovery Research	Amendment
1	DCMC	New
2	Technology Platform Program Pilot	New
3	CLIN4 Updates	Amendment
3	Rare Disease Platform Pilot	New
4	Preclinical Development	New
4/5	CLIN2 Updates	Amendment
6	EDUC Training Program Updates	Amendment
6	EDUC Hybrid Skillset Training Program	New
Other	EDUC1 Conference Grant Updates	Amendment

Concept Development

Timeline

➢ **October-December:** Define initial program frameworks and align across programs

➢ **Early January:** Develop Program Announcements and draft concepts

➢ **Late January:** Socialize high-level concepts with Committee Chairs; revise concepts and program announcements based on feedback

➢ **February:** Re-review concepts with Committee Chairs; revise further based on feedback

➢ **Early March:** Present at Science Subcommittee for recommendation to move forward to ICOC

➢ **Late March:** Present at ICOC for approval

Program Announcement and Application

Timeline

➢ **Late January:** Initial application framework defined

➢ **Late February:** Submit GMS requirements to IT for system updates

➢ **Late March:** Complete application templates and finalize review module specs

➢ **April:** Finalize and post Program Announcements

➢ **April:** Conduct application beta testing

➢ **May:** Launch application system

➢ **June:** Launch reviewer module and deliver GWG training materials

Ongoing: GWG recruitment (Jan–June) and training (TBD)

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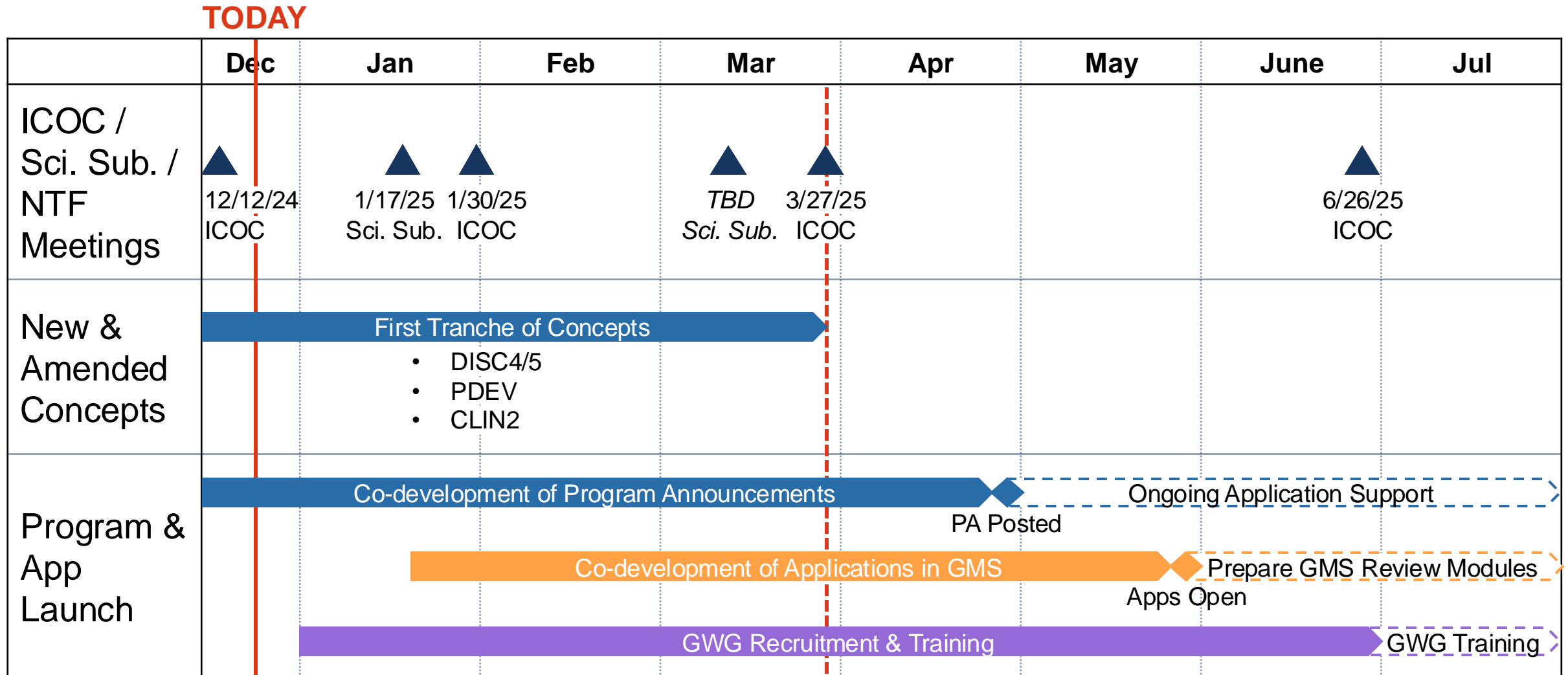
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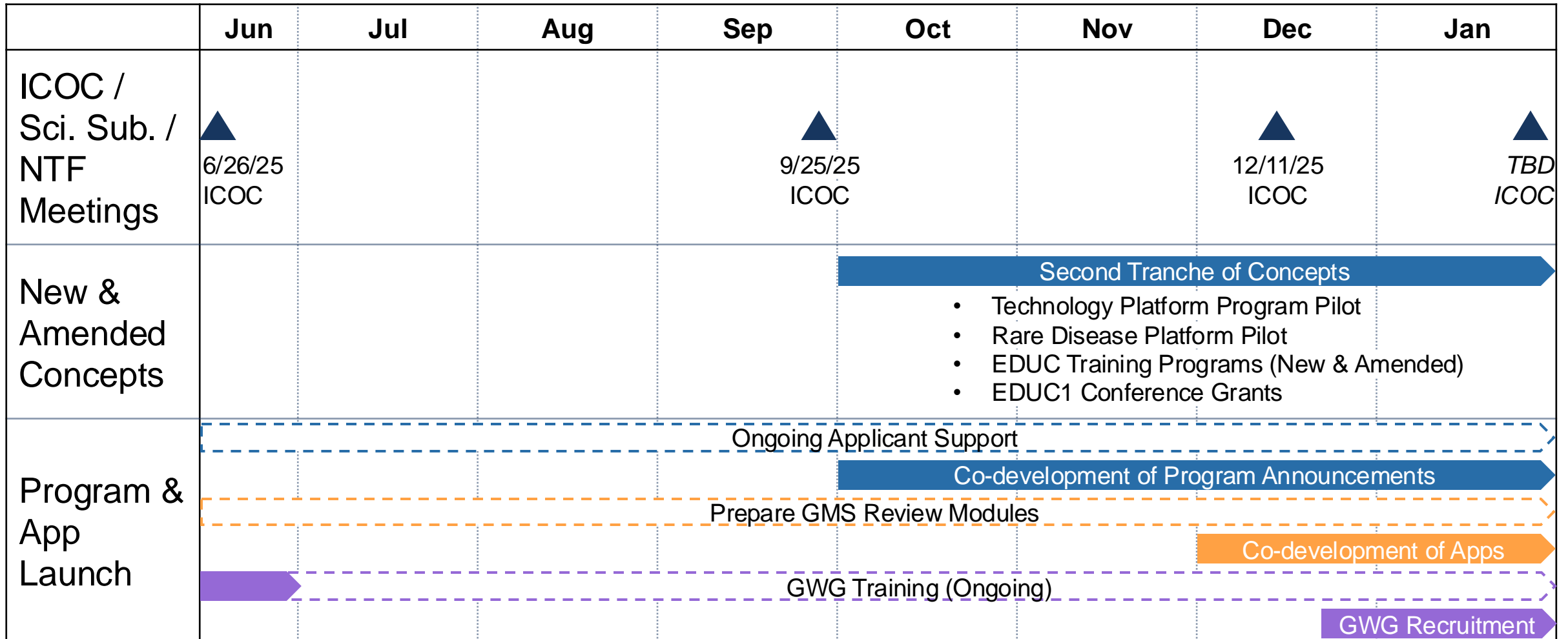
3   Concept Development Timeline

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SAF Implementation Timeline (2/2)

	Jun	Jul	Aug	Sep	Oct	Nov	Dec	Jan
ICOC / Sci. Sub. / NTF Meetings	▲ 6/26/25 ICOC			▲ 9/25/25 ICOC			▲ 12/11/25 ICOC	▲ *TBD ICOC*
New & Amended Concepts					Second Tranche of Concepts → • Technology Platform Program Pilot • Rare Disease Platform Pilot • EDUC Training Programs (New & Amended) • EDUC1 Conference Grants			
Program & App Launch	GWG Training (Ongoing) →	Ongoing Applicant Support			Co-development of Program Announcements → Prepare GMS Review Modules		Co-development of Apps → GWG Recruitment →	

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Timeline | Concept Development & Amendments

Goal	Concept	Type	Tranche & ICOC Meeting
1	Revised DISC4/5 for Discovery Research	Amendment	1 - March 2025
1	DCMC	New	TBD
2	Technology Platform Program Pilot	New	2 - Q3/4 FY25-26
3	CLIN4 Updates	Amendment	3 - Q1 FY26/27
3	Rare Disease Platform Pilot	New	2 - Q3/4 FY25-26
4	Preclinical Development	New	1 - March 2025
4/5	CLIN2 Updates	Amendment	1 - March 2025
6	EDUC Training Program Updates	Amendment	2 - Q3/4 FY25-26
6	EDUC Hybrid Skillset Training Program	New	2 - Q3/4 FY25-26
Other	EDUC1 Conference Grant Updates	Amendment	2 - Q3/4 FY25-26

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