

Strategic Allocation Framework – Implementation Update

Jonathan Y. Thomas, J.D., Ph.D.,
ICOC Meeting
December 12th, 2024



Presentation Overview

1 SAF Recap: Goals & Recommendations

2 SAF Planning to SAF Implementation

3 Concept Development Timeline

4 Questions

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
<p>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</p>	<p>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</p> <hr/> <p>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</p>
<p>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</p>	<p>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</p>

Summary | SAF Goals & Recommendations (2/4)

Goals	Recommendations
<p>3. Advance 4-7 rare disease projects to BLA</p>	<p>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</p> <hr/> <p>Pilot Platform-Based Therapy Development Implement pilot platform-based approach for gene therapy development using life-threatening monogenic neurological disorders as a test case</p>
<p>4. Propel 15-20 therapies targeting diseases affecting Californians to late-stage trials</p>	<p>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</p> <hr/> <p>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</p>

Summary | SAF Goals & Recommendations (3/4)

Goals	Recommendations
<p>5. Ensure that every BLA-ready program has a strategy for access and affordability</p>	<p>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</p> <hr/> <p>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</p> <hr/> <p>Influence Policy Deploy AAWG resources to advocate for policies that advance access & reimbursement for regenerative medicines</p> <hr/> <p>Enhance Partnerships Engage state & national partners to align initiatives that expand sustainable access to regenerative medicines</p>

Summary | SAF Goals & Recommendations (4/4)

Goals	Recommendations
<p>6. Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine</p>	<p>Provide high-demand technical training via Bridges & COMPASS program updates Increase training offerings, diversify internship types, & increase integration with CIRM R&D grants</p> <hr/> <p>Create new EDUC program to develop hybrid skillsets Implement new program structure to focus on cross-disciplinary internships</p> <hr/> <p>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</p>
<p>Additional Recommendations</p>	<p>Restart Grantee Conference to Report SAF Goal Progress Restart recurring grantee conference (timing TBD) with main objective of reporting progress on SAF goals</p> <hr/> <p>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</p>

Presentation Overview

1 SAF Recap: Goals & Recommendations

2 SAF Planning to SAF Implementation

3 Concept Development Timeline

4 Questions

SAF Planning

SAF Implementation

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)

Presentation Overview

1 SAF Recap: Goals & Recommendations

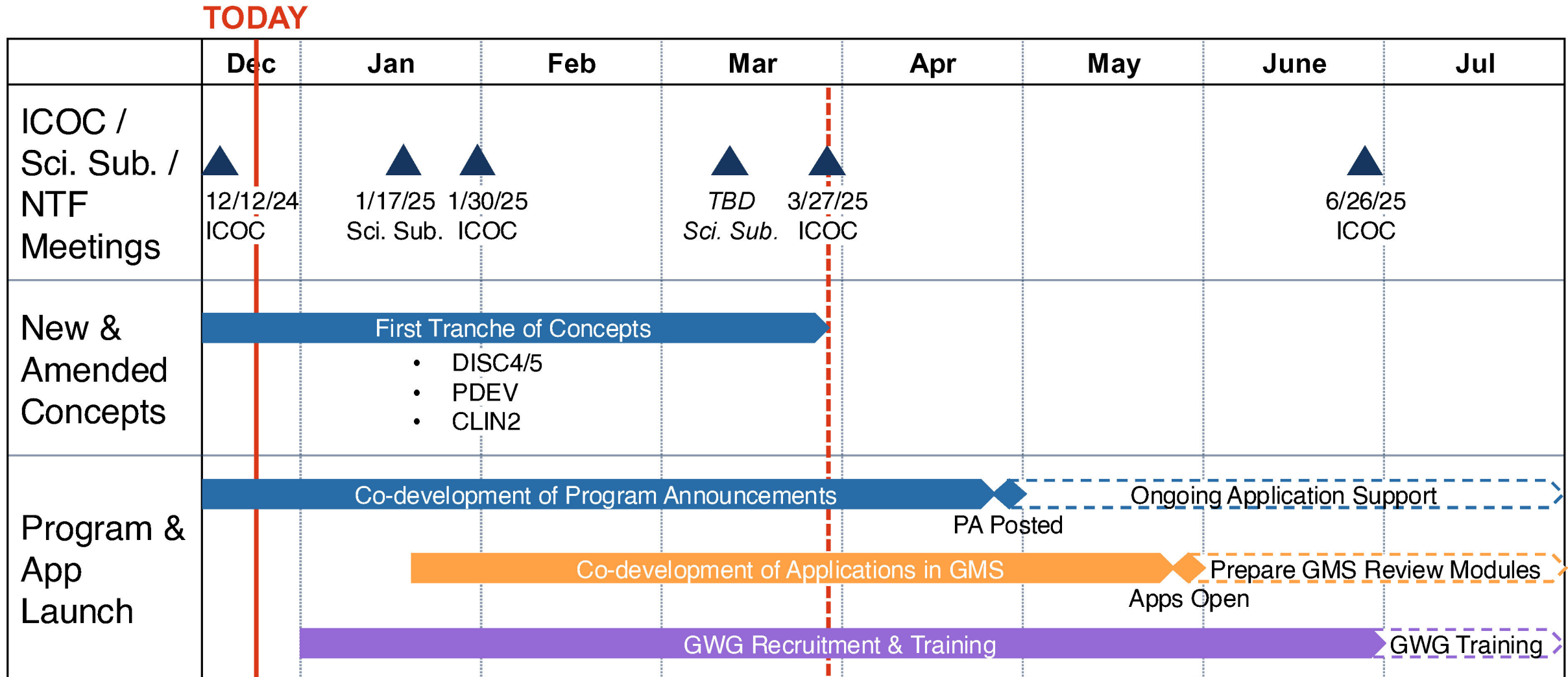
2 SAF Planning to SAF Implementation

3 Concept Development Timeline

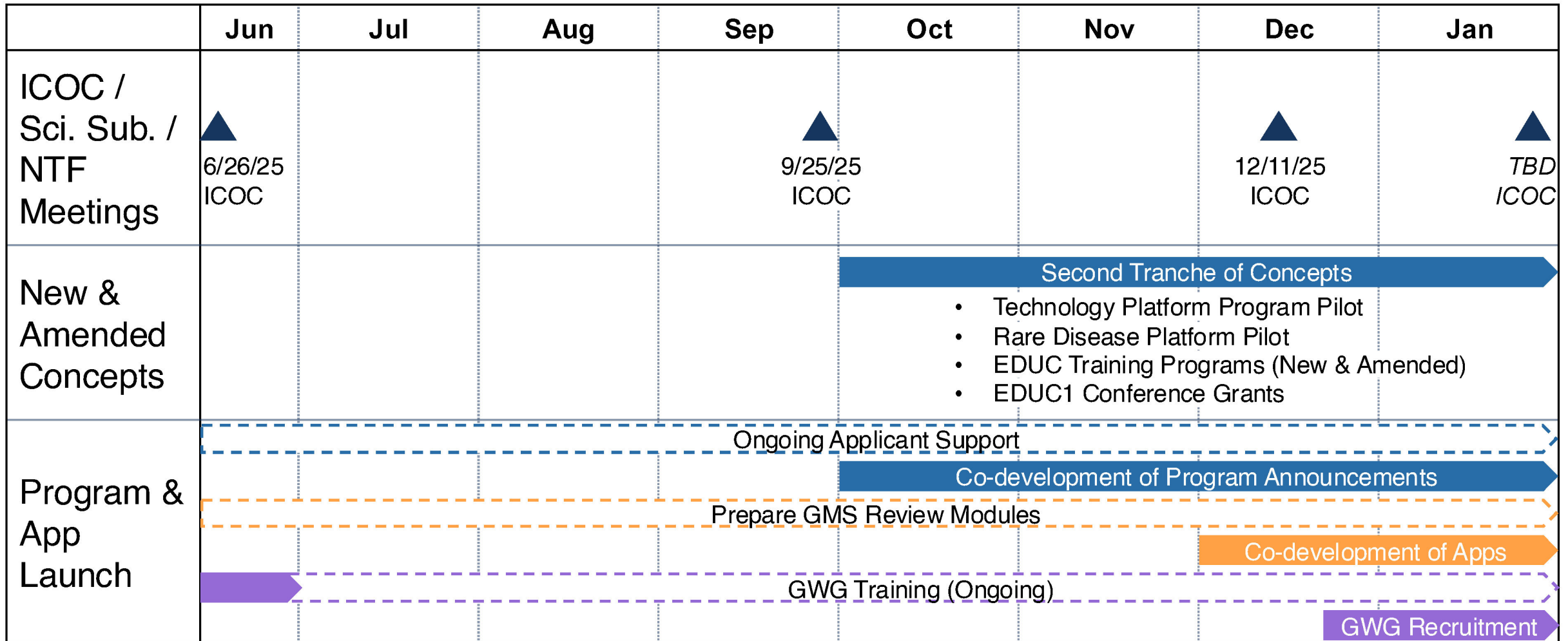
4 Questions

SAF Implementation Timeline (1/2)

TODAY



SAF Implementation Timeline (2/2)



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

Presentation Overview

1 SAF Recap: Goals & Recommendations

2 SAF Planning to SAF Implementation

3 Concept Development Timeline

4 Questions

Questions