Joint Science Subcommittee / Neuro Task Force Meeting

September 13, 2024

CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Actions Today

1. Review & discuss Goals 5 & 6
2. Recap Goals 1-4
3. Obtain endorsement of Goals & Recommendations

Presentation Overview

1. Pre-read: Background
2. Pre-read: SAF Overview
3. Goal 5
4. Goal 6
5. Additional Recommendations
6. Updates to Goals 1 & 2
7. Updates to Goals 3 & 4
8. Discussion/Next Steps

Please note:

To ensure ample time for discussion, the Background and SAF Overview will not be presented during the meeting on August 16th. For those interested, these sections were previously presented at the June 27th ICOC meeting. Please review these slides accordingly. (6:52:15 timepoint)

Goals 1 & 2 were presented at the July 11th Joint Science Subcommittee & Neuro Task Force Meeting. (2:38 timepoint)

Goals 3 & 4 were presented at the August 16th Joint Science Subcommittee & Neuro Task Force Meeting. (3:00 timepoint)

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5-Year Strategic Plan (2022-2027) | 3 Themes

Advance World Class Science

- Develop shared resources
- Build knowledge networks

Deliver Real World Solutions

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

Provide Opportunity for All

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

Background & Rationale

CIRM must allocate remaining resources to maximize its impact by considering available funds and reviewing past strategies

- CIRM has established itself as a leader in stem cell and regenerative medicine, funding basic research, infrastructure, education/training, and regenerative medicine discovery and clinical development
- Since CIRM's inception, the regenerative medicine field has grown exponentially
- CIRM has finite resources
- Demand for CIRM funding exceeds available resources

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SAF Mandate

- September 2023 Science Subcommittee: Prioritization Kickoff Discussion (BM Fischer-Colbrie)
  - **Outcome:** Ask for CIRM staff to develop an approach and recommendations for prioritization
- March 2024 Science Subcommittee and ICOC: Presented SAF and continued process with September 2024 target for recommendations

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

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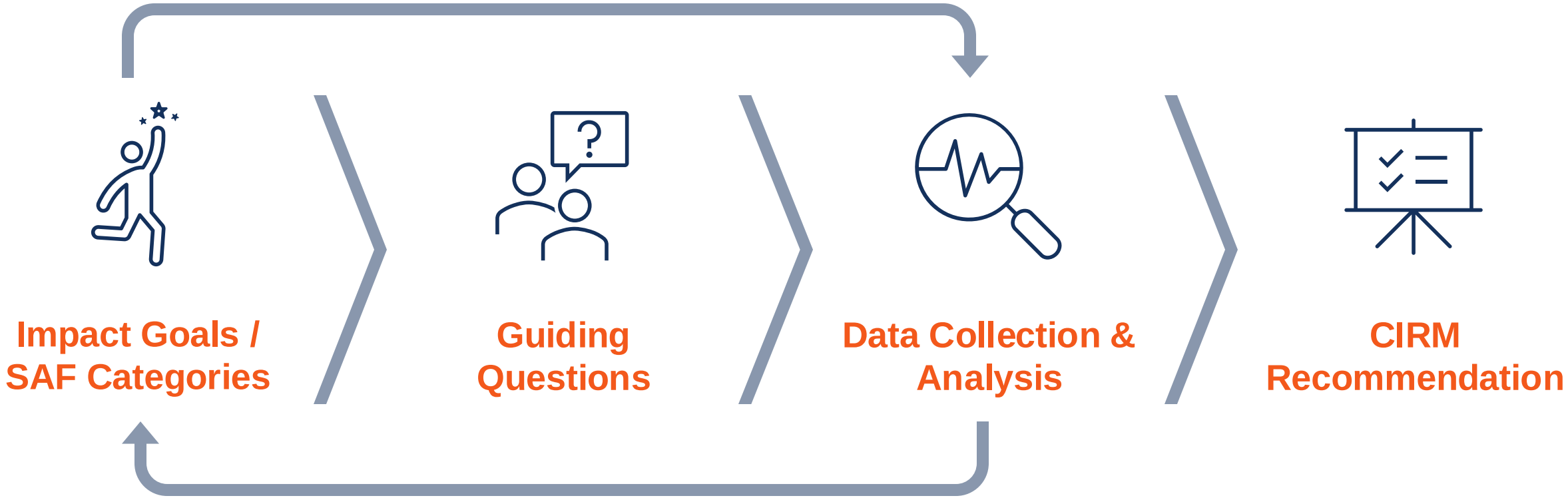
SAF | Design Questions

Determine:

- How can CIRM make the greatest impact on its mission?
- How might CIRM effectively allocate its remaining budget of $3.86B?
  - How might CIRM effectively allocate its remaining Neuro budget of $1.14B?

SAF | Process*

CIRM Staff



*Science Subcommittee, NTF, AAWG will inform specific aspects of the Recommendations

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SAF Categories

SAF Categories

1. Discovery of Novel Disease Mechanisms
2. Cell and Gene Therapy Approvals
3. Accessibility and Affordability of CIRM-Funded Cell and Gene Therapies
4. Diverse Workforce Development

*NTF will inform specific aspects of the Recommendations

CIRM Preliminary* Impact Goals

Accelerating Discovery & Translation

1. **Catalyze** the identification and validation of at least X novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California
2. **Accelerate** development and utilization of X technologies that demonstrate improvements in safety, efficacy, and quality of cell and gene therapies

Cell & Gene Therapy Approvals

3. **Advance** at least X rare disease projects to BLA
4. **Propel** X therapies targeting diseases affecting Californians to late-stage trials

Accessibility & Affordability of CIRM-Funded Cell & Gene Therapies

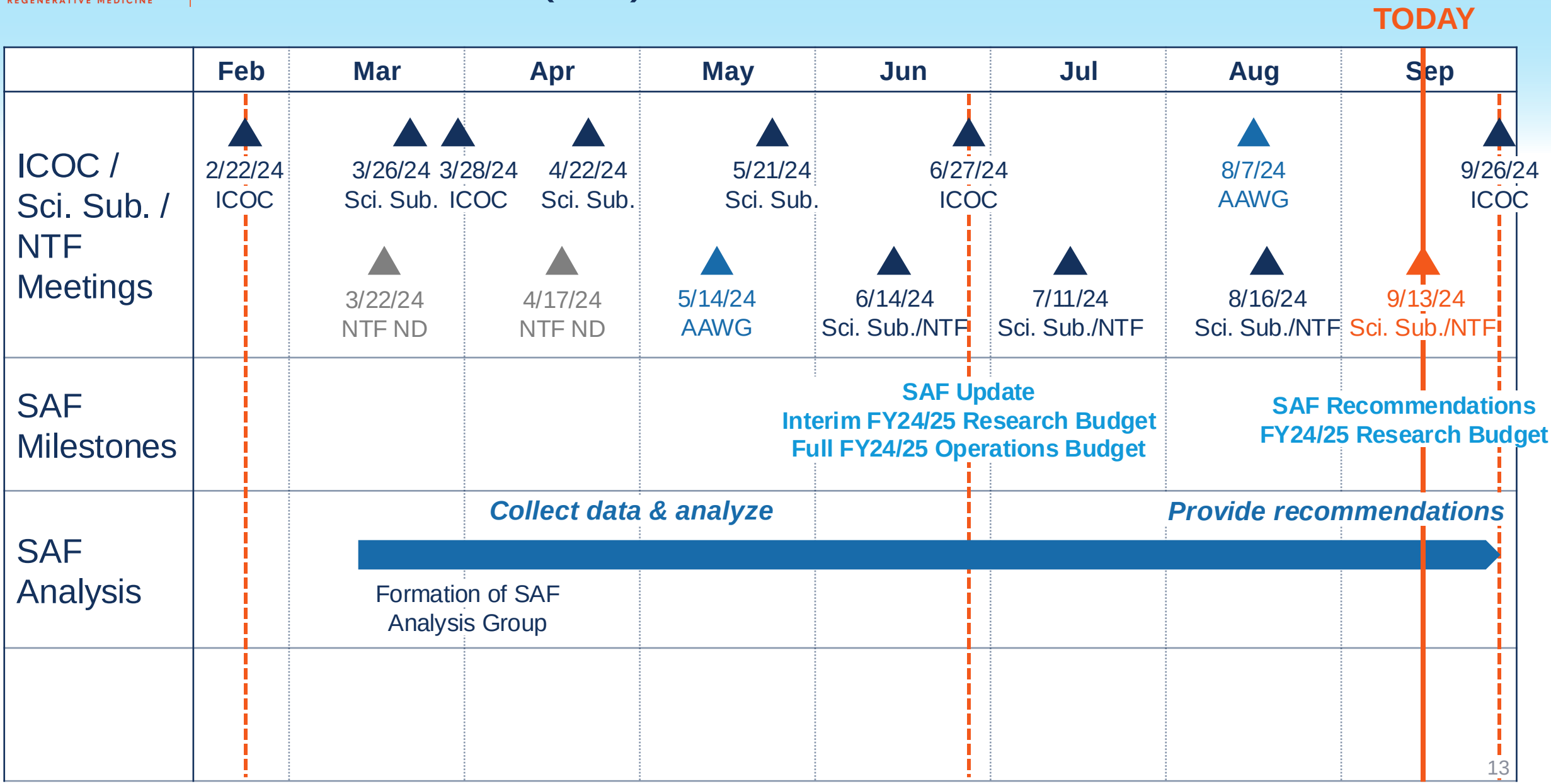
5. **Ensure** that every BLA-ready program has a strategy for access and affordability

Diverse Workforce Development

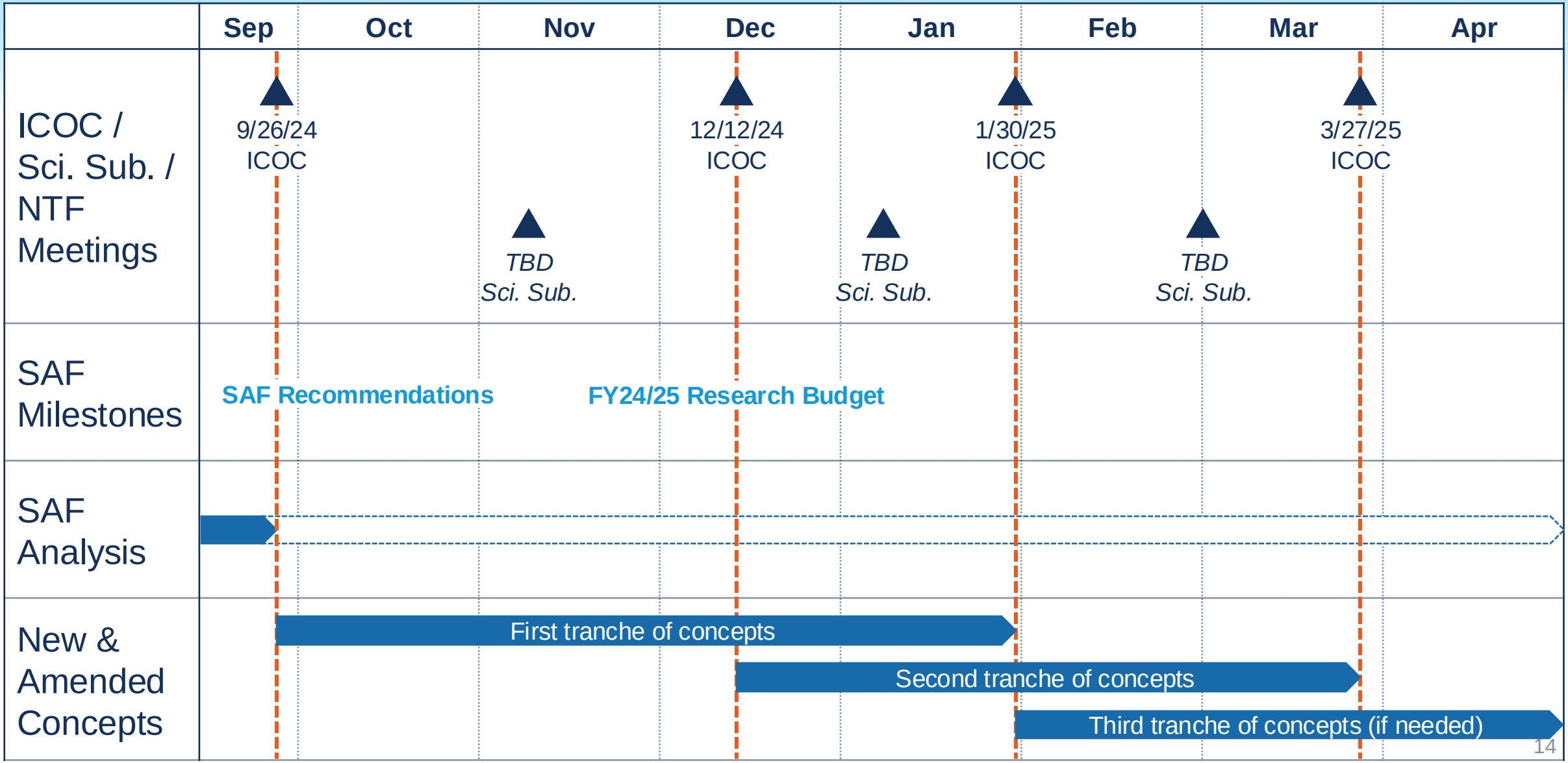
6. **Bolster** CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

**Preliminary Impact Goals to be refined through September ICOC*

SAF Timeline (1/2)



SAF Timeline (2/2)



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Preliminary Goal 5

Category: Accessibility & Affordability of CIRM-Funded Cell & Gene Therapies

Goal 5 - Ensure that every BLA-ready program has a strategy for access and affordability

Preliminary Goal 5 | High-Level Questions

Goal 5 - Ensure that every BLA-ready program has a strategy for access and affordability

High-Level Questions

- **Landscape**
  - What are the most impactful factors for achieving access and affordability?
  - What are the barriers to access and affordability for CGTs?
  - What research is needed to understand the landscape for access and affordability?
- **CIRM Program Enhancements**
  - What activities within CIRM could be developed to facilitate access and affordability?
  - At what stage should the applicants provide an access and affordability strategy?
  - How can strategies be scaled if the therapy is successful?
- **External Engagements**
  - Who are the most important partners to impact policy change?

Goal 5 | Data Sources

- **Portfolio Data** (as of February 2024)
- **AAWG Considerations** (May & August 2024)
- **GWG Recommendations:** CLIN2 awards (2023)
- **Centers for Medicare & Medicaid Services (CMS)**: Hospital Inpatient PPS final rule (FY 2024)
- **ICER (Institute for Clinical and Economic Review)/NEWDIGS (New Drug Development Paradigms) white papers:** Accessibility of CGT approved therapies (2024)

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CIRM's Clinical Infrastructure Programs

Program	Overview	Barriers to Access				
		Clinical Expertise	Cohort Dev	Geography	Patient Knowledge	Financial
Alpha Clinics	• Launched 2015 • Support CGT clinical trials • 275+ clinical trials (71 CIRM-funded)	✓	✓			
Community Care Centers of Excellence (CCCEs)	• Launching 2025 • Support patient access to clinical trials		✓	✓	✓	
Patient Support Program (PSP)	• Launching 2025 • Address financial & logistical needs of patients in CIRM-funded trials			✓	✓	✓

Patient Access Programs are nascent, but aim to reduce patient barriers to clinical trials

Source: CIRM Portfolio as of February 2024

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CGT Access Challenges

Approved CGT Access Challenges
Limited clinical evidence generated prior to approval to inform long-term efficacy & durability vs. SOC
Very high initial cost of treatment compared to small molecules or biologics
Necessity of specialized treatment centers for delivery of treatment
Variability in coverage & reimbursement rates across Medicare, Medicaid, private insurers
Complex manufacturing & supply chains, particularly for autologous gene-modified cell therapies

Source: Tunis, et. Al., Health Policy (2021); “Managing the Challenges of Paying for Gene Therapy: Strategies for Market Action and Policy Reform” ICER & NEWDIGS

Goal 5 | Objective & Approach

Goal 5 - Ensure that every BLA-ready program has a strategy for access and affordability

- **Objective**: Promote accessibility & affordability of CIRM-funded therapeutics to all California patients during clinical trials & beyond
- **Approach**:
  - Leverage CIRM Clinical Infrastructure and resource Clinical Trial programs to achieve enrollment objectives and stage-appropriate access planning
  - Influence policies that impact access and affordability through advocacy partnerships

Goal 5 | Recommendations (1/2)

Goal 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

Goal 5 | Recommendations (2/2)

Goal 5 - Ensure that every BLA-ready program has a strategy for access and affordability

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

CGT Access Challenges & CIRM Programs

3 Goal 5 Recommendations

Approved CGT Access Challenges	CIRM Programs & Initiatives
Limited clinical evidence generated prior to approval to inform long-term efficacy & durability vs. SOC	**Update CLIN2** to incentivize access strategy development and provide **AAWG support**
Very high initial cost of treatment compared to small molecules or biologics	**Patient Assistance Fund** will support access to CIRM-funded treatments
Necessity of specialized treatment centers for delivery of treatment	Expand capacity through **CCCE-Alpha Clinic partnerships**
Variability in coverage & reimbursement rates across Medicare, Medicaid, private insurers	Engage **policy partners** & deploy AAWG resources for **advocacy**
Complex manufacturing & supply chains, particularly for autologous gene-modified cell therapies	**Technology Platform Program & Manufacturing Network** will address manufacturing bottlenecks

Source: Tunis, et. Al., Health Policy (2021); "Managing the Challenges of Paying for Gene Therapy: Strategies for Market Action and Policy Reform" ICER & NEWDIGS

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Preliminary Goal 6

Category: Diverse Workforce Development

Goal 6 - Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

Preliminary Goal 6 | High-Level Questions

Goal 6 - Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

High-Level Questions

- **Identifying Competency Gaps**
  - What competencies are currently lacking in the CGT workforce?
  - What types of roles or positions are most in demand for these areas of need?
  - What training is needed for these competencies, and how/where is it obtained?
- **Increasing Diversity/Representation**
  - What groups have challenges to enter and stay in the CGT workforce?
  - How can these challenges be addressed through education and training?
  - How can CIRM increase availability of opportunities to these groups?
- **Leveraging Collaborations & Best Practices**
  - What synergies exist between CIRM's EDUC and other Infrastructure programs?
  - How can CIRM leverage investments and external infrastructure/resources to expand the reach and scope of EDUC programs?

Goal 6 | Data Sources

- **Biotech industry workforce gap analyses, forums, and reports**: TEConomy, California Economic Impact Report, Biotechnology Skilled Needs Assessment
- **Cell and Gene Therapy workforce analysis reports**: Nature Biotech, NIMBL, Alliance for Regenerative Medicine, National Academy of Sciences
- **CIRM internal portfolio and trainee analysis** (2009-present)
- **Research in hybrid skillset training needs**
  - Research articles
  - Peer review papers
- **Research in CA education landscape**: CA Department of Education, US Census Bureau, CA Commission on Teacher Credentialing
- **Demographic and diversity reports**
  - University of California Information Center
  - The California State University Enrollment
  - California Community Colleges Research and Data Analytics
  - Reports on diversity in biotech industry
- **Meetings with education stakeholders**

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Competencies vs. Training Availability

Competency	Academic Training in CA	CIRM EDUC/INFR Training						
		SPARK	COMPASS	Bridges	Scholars	Manf	Alpha	SRL
Process Development	Limited		✓	✓	✓	✓		
Manufacturing	Limited		✓	✓		✓		
Quality Assurance/Control	Limited		✓	✓		✓		
Data Science for Bio	Some		✓	✓	✓			
Research, General	Many	✓	✓	✓	✓			
Research, CGT	Some	✓	✓	✓	✓			
Clinical Research	Limited		✓	✓			✓	
Stem Cell Modeling	Some	✓	✓	✓	✓			✓

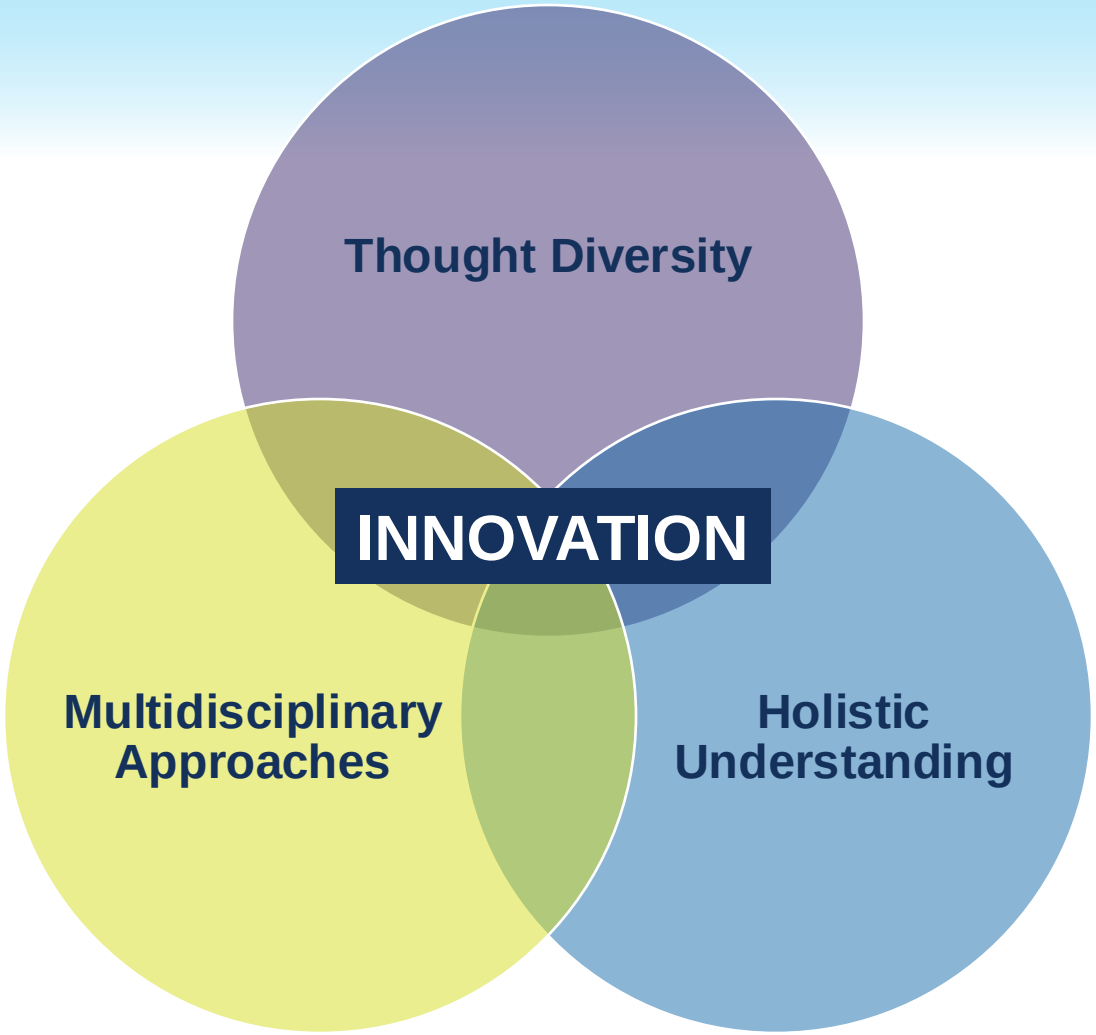
✓ = Some trainees get this opportunity; ✓ = Most trainees get this opportunity

Sources: ARM, WORKFORCE GAP ANALYSIS FOR THE CELL AND GENE THERAPY SECTOR MARCH 2023; CIRM Portfolio Analysis; CA Community College Centers of Excellence Report, 2022

Hybrid Skill Sets Are Rare But Critical For Innovation

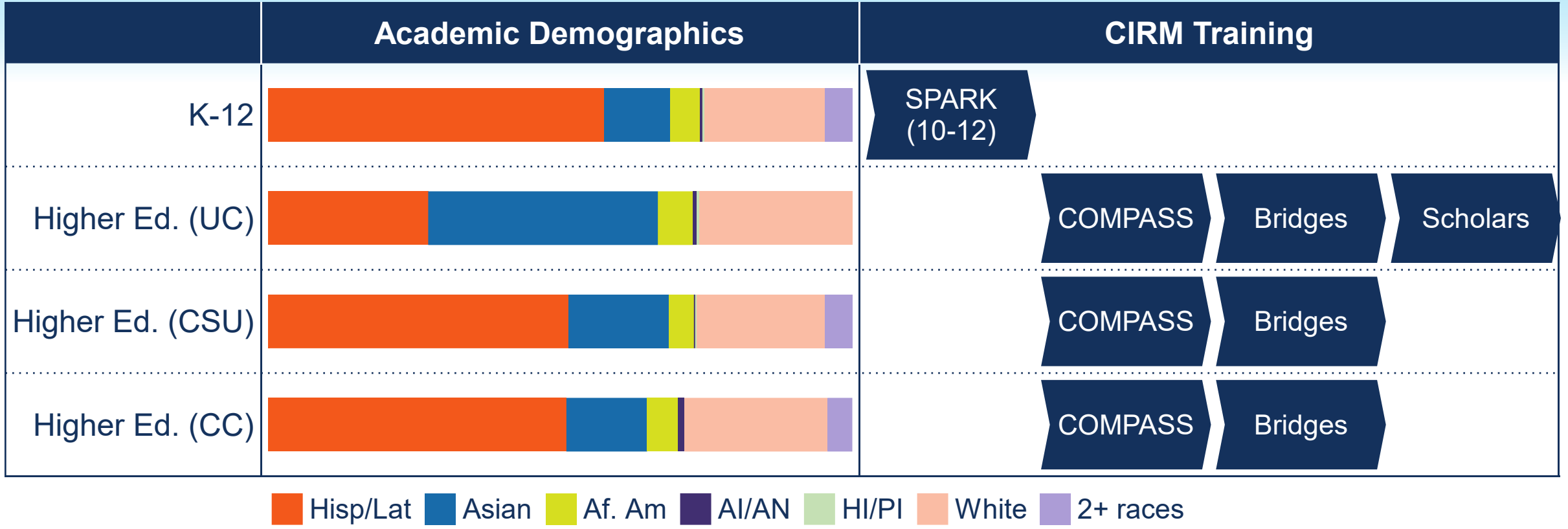
- Silos between disciplines & industries stifle innovation and limit diversity of thought
- Increased holistic understanding improves efficiency

Individuals with hybrid skill sets are highly valued for overcoming roadblocks and driving innovation



Sources: Notes from NAS Regenerative Medicine Forum, Workforce Discussion June 4-5, 2024 (Proceedings in preparation); Post et al., Capitalizing on thought diversity for innovation, Routledge, Taylor and Francis Group; 2023 Life Science TEConomy Report; NAS Press: Research at the Intersection of the Physical and Life Sciences (2010)

Demographic Attrition Begins Prior to College



Targeted and consistent outreach earlier in education (K-10th grade) is needed to prevent drop of underrepresented students in higher education

Sources: UC Accountability Report 2022, CSU Demographic Report, Education Trust West CA STEM by the Numbers 2015 report, CA Commission on Teacher Credentialing

Data Analysis | Summary

- **Identifying Competency Gaps**
  - Limited exposure/accessibility to manufacturing and clinical career paths in CA academic training
  - Few opportunities for:
    - Hands-on training in development, manufacture, and translation of CGT
    - Developing cross-disciplinary skillsets
- **Increasing Diversity/Representation**
  - Some populations continue to be underserved and underrepresented in the workforce
  - Attrition of diverse perspectives begins prior to college entry
- **Leveraging Collaborations & Best Practices**
  - Opportunities to increase connectivity and intra-program collaboration

Goal 6 | Objective & Approach

Goal 6 - Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

- **Objectives**:
  - Increase access to in-demand CGT workforce competencies that are currently limited in academic training environments
  - Increase diversity of the future CGT workforce

- **Approaches**:
  - Enhance scope of CIRM's core EDUC programs
  - Implement outreach and education campaigns to introduce regenerative medicine concepts & career possibilities to California's diverse communities

Goal 6 | Recommendations

Goal 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

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Additional Recommendations

Additional recommendations to support all goals

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

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Goals 1 & 2

Category: Accelerating Discovery & Translation

Goal 1 - Catalyze the identification and validation of at least X novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California

Goal 2 - Accelerate development and utilization of X technologies that demonstrate improvements in safety, efficacy, or quality of cell and gene therapies

Goal 1 Recommendations

Goal 1 - Catalyze the identification and validation of at least X 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

Recommendations for Goal 2

Goal 2 - Accelerate development and utilization of X technologies that demonstrate improvements in safety, efficacy, 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

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Preliminary Goals 3 & 4

Category: Cell & Gene Therapy Approvals

Goal 3 - Advance at least X rare disease projects to BLA

Goal 4 - Propel X therapies targeting diseases affecting Californians to late-stage trials

Goal 3 | Recommendations

Goal 3 - Advance at least X 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

*Not exclusive to rare disease, but majority of programs near BLA in CIRM's portfolio are in rare disease

Goal 4 | Recommendations

Goal 4 - Propel X 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**

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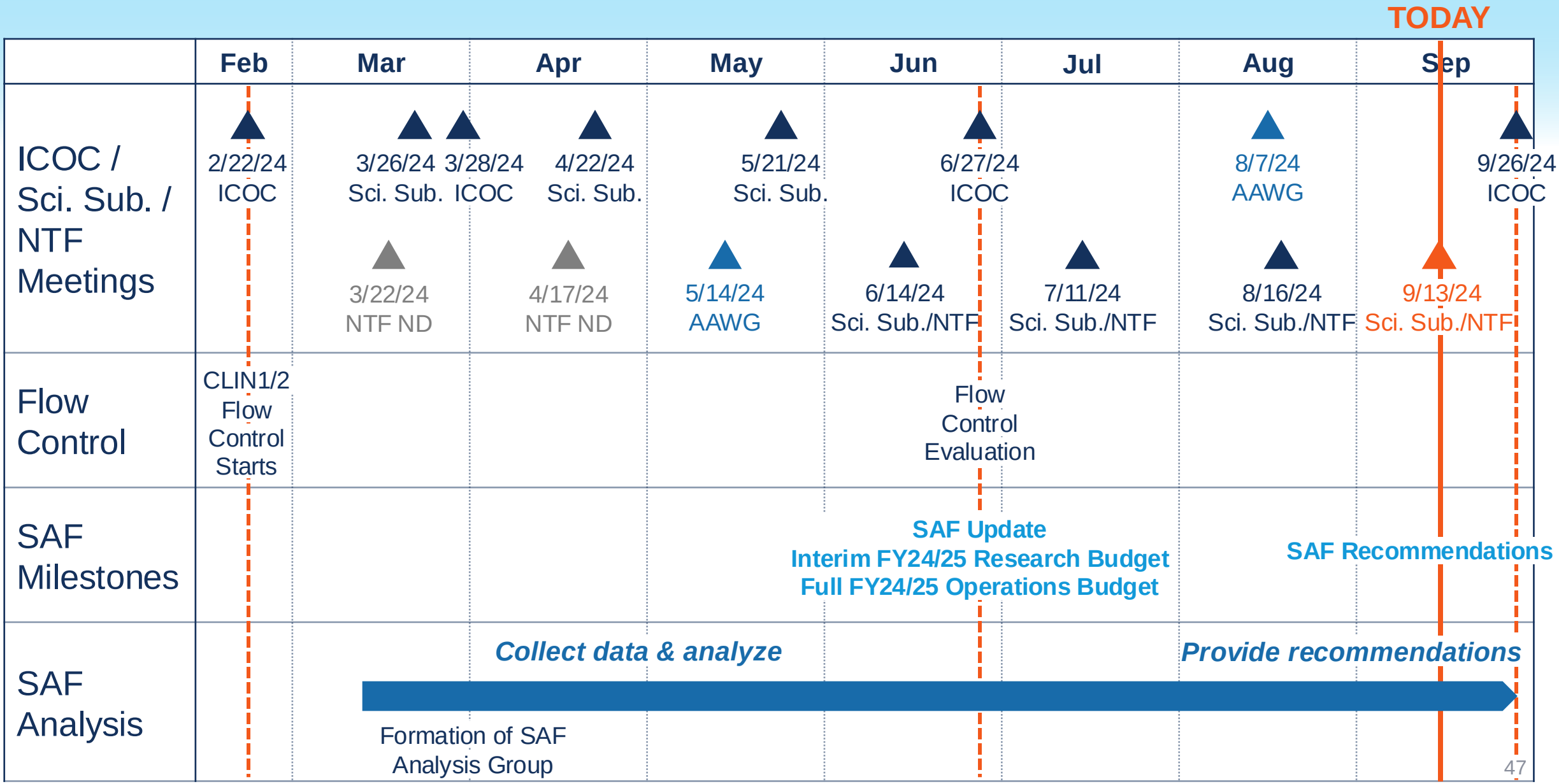
Timeline & Next Steps

Meeting	SAF Topics
June NTF/Science Subcommittee	• SAF Overview - NTF Background • Present Neuro Survey Results – Discussion • Provide a high-level overview of how this fits within Strategic Analysis Framework (SAF)
June ICOC	• Provide an update on the process, aligning with the June NTF/Science Subcommittee • Offer an example of analysis that will inform recommendations
July NTF/Science Subcommittee	• Present four overarching SAF Goals and delve into Goals 1 & 2 • Review relevant data associated with Goal 1 & 2 • Discuss potential recommendations for Goal 1 & 2
August AAWG	• Present updates on Goal 5 • Discuss considerations for Goal 5
August NTF/Science Subcommittee	• Present updates based on feedback received on Goal 1 & 2 • Introduce Goal 3 & 4 and discuss associated data • Discuss potential recommendations for Goals 3 & 4

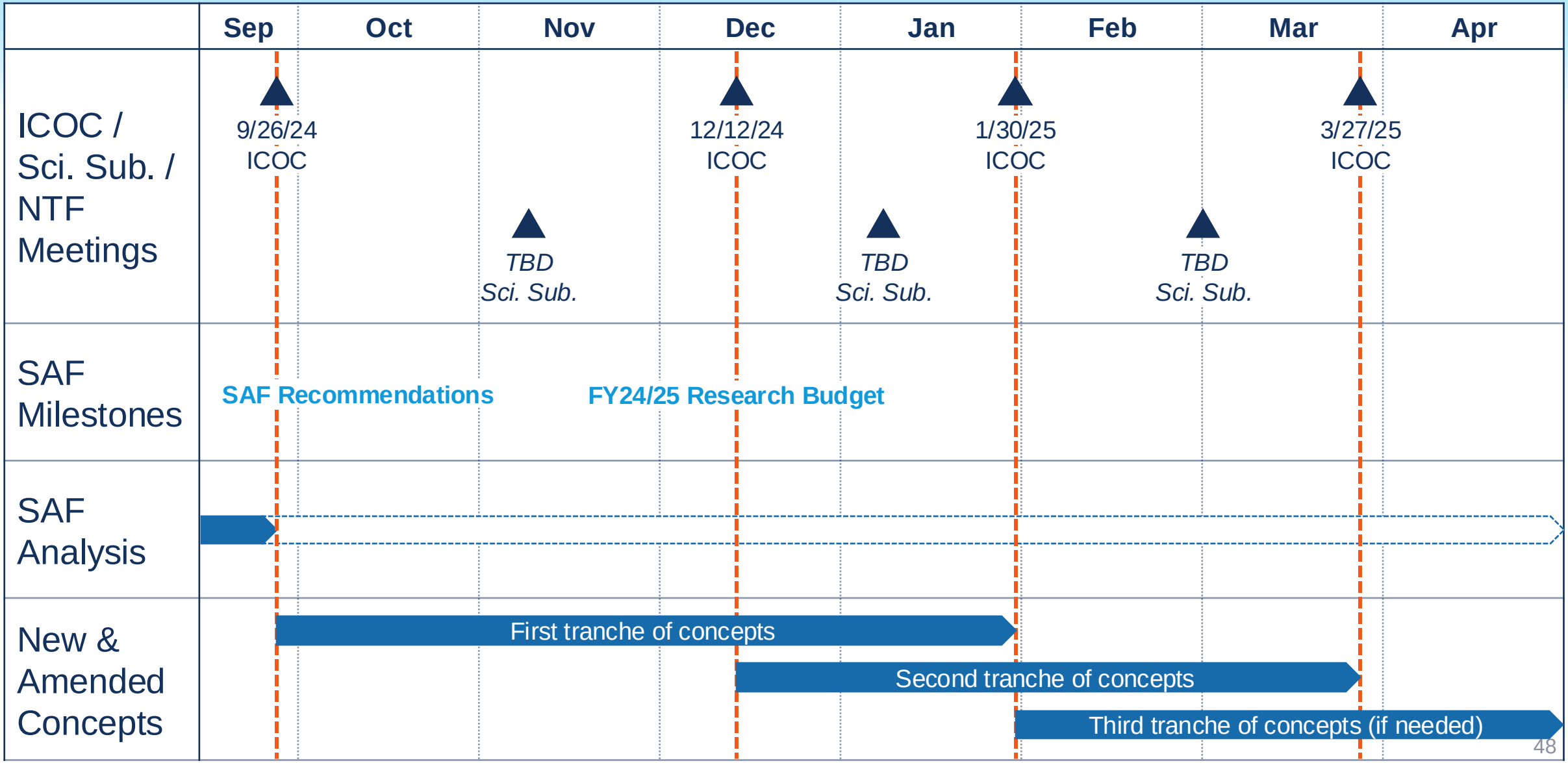
September NTF/Science Subcommittee	• Full SAF presentation:
	  • Present updates based on feedback received on Goals 1, 2, 3, & 4
	  • Present Goals 5 & 6
	  • Discuss overall recommendations in preparation for September ICOC

September ICOC	• Overall Presentation of SAF recommendations

SAF Timeline (1/2)



SAF Timeline (2/2)



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Summary | SAF Goals & Recommendations (1/3)

Goals	Recommendations
1. Catalyze the identification and validation of at least X 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 X technologies that demonstrate improvements in safety, efficacy, 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
3. Advance at least X 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

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Summary | SAF Goals & Recommendations (2/3)

Goals	Recommendations
4. Propel X 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
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

CIRM CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Summary | SAF Goals & Recommendations (3/3)

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

Requested Action

We request a motion that the Science Subcommittee/Neuro Task Force recommend approval to the full board of these goals and recommendations

Glossary of Terms

- **BLA**: Biologics License Application
- **Master Protocol**: A clinical trial protocol designed with multiple coordinated sub-studies to evaluate one or more investigational drugs for one or more diseases within the overall trial structure
  - **Basket Trial**: A master protocol designed to study a single investigational drug in multiple diseases or disease subtypes
  - **Platform Trial**: A master protocol designed to study multiple investigation drugs in a single disease in a perpetual manner, with therapies allowed to enter or leave the platform based on a decision algorithm
  - **Umbrella Trial**: A master protocol designed to study multiple investigational drugs in the context of a single disease
- **Platform Technology**: A technology that can be incorporated in multiple therapies or that can be used for the research, development and/or manufacture of multiple therapies.
- **Rare Disease**: A disease with a prevalence of <200,000 patients in the US
  - **Ultra-rare Disease**: A disease with a prevalence of <10,000 patients in the US