

August 2024 AAWG

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C I R M
CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE

Patient Access Updates

1. Patient Support Program Implementation
2. Community Care Centers of Excellence RFA
3. NIH RFI Access Plans

Strategic Allocation Framework

1. Background
2. SAF Overview
3. Goal 5
4. Discussion

- **Blanca Batlle** CIRM Program Manager Patient Support
- CIRM Eversana Kickoff Meeting 4/16/24
- CIRM Eversana meeting weekly
- Aiming for program pilot Q4-24 – Q1-25

- **Emily Crotti** CIRM Project Manager Patient Support
- CIRM sponsored two webinars
- CIRM engagement with applicants
- Most applicants have engaged Alpha Clinic sites
- Applications due August 15, 2024

- Proposal within its Intermural Research Program intended to promote access
 - Includes drugs, biologics, vaccines or devices
- CIRM team and AAWG members provided feedback/comments
- Holds potential to inform access planning in the future

Strategic Allocation Framework

Goal 5

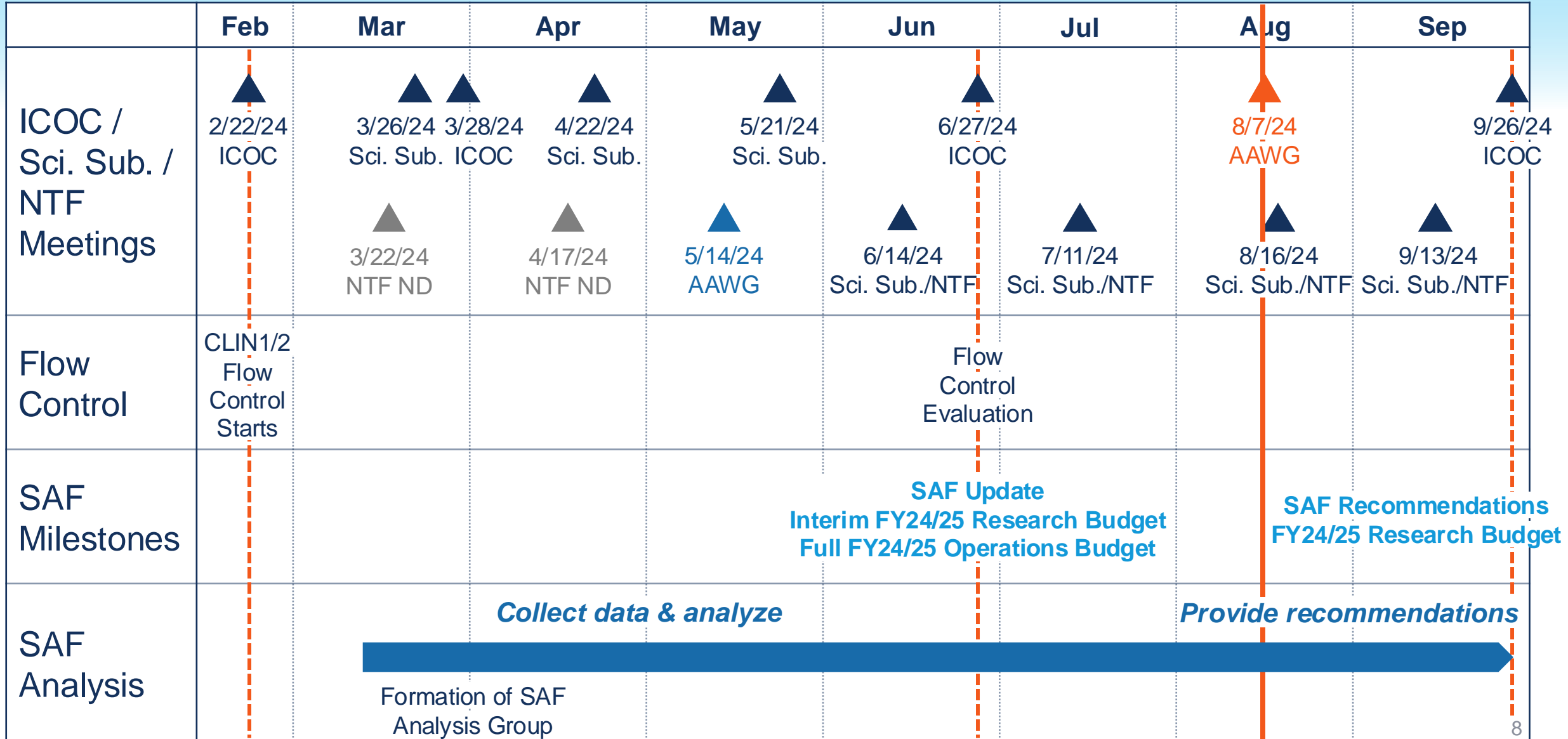
- 1 Background
- 2 SAF Overview
- 3 Goal 5
- 4 Discussion/Next Steps

Please note:

To ensure ample time for discussion, the Background and SAF Overview will not be presented during the meeting on August 7th. For those interested, these sections were previously presented at the June 27th ICOC meeting. Please review these slides accordingly.

[\(6:52:15 timepoint\)](#)

TODAY



1 Background

2 SAF Overview

3 Goal 5

4 Discussion/Next Steps

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

- 5. Ensure** that **every** CIRM funded project completing a late-stage clinical trial has a strategy that enables access and affordability by all California patients, particularly underserved populations

Review Preliminary Goal 5:

1. High-Level Questions
2. Considerations*
3. Discussion

5. Ensure that **every** CIRM funded project completing a late-stage clinical trial has a strategy that enables access and affordability by all California patients, particularly underserved populations

High-Level Questions

1. How will CIRM align with CMS cell and gene therapy access model?
2. What are the most impactful factors for achieving access and affordability?
3. What research is needed to understand the landscape for access and affordability?
4. What general policies within CIRM could be developed to facilitate access and affordability?
5. What are the barriers to access and affordability for CGTs?
6. At what stage should the applicants provide an access and affordability strategy?
7. How can strategies be scaled if the therapy is successful?
8. What are additional opportunities for access and affordability?

Considerations for Recommendations

- Support CMS inclusion for CIRM-funded sickle cell gene therapy programs by demonstrating cost, efficacy, durability, and safety
- Continue engaging DHCS to build infrastructure supporting NOFO applications
- Engage Alpha Clinics and CCCE Steering Committees in partnership opportunities
- Prioritize durability assessment for CIRM CLIN programs' coverage decisions
- Develop support systems for researchers on health economics and outcomes research (HEOR)
- Document CGT program costs to reduce disparities in access and care
- Evaluate tools like ICER-Analytics for comparative cost-effectiveness
- Leverage NIH policy framework for access and affordability strategies
- Identify barriers and solutions through surveys/research on CIRM CLIN programs
- For Rare Disease Programs, consider sharing financial risks between sponsors, manufacturers, and CIRM
- For CIRM-funded rare disease programs, use CAP/MAP processes to advise on actuarial risk and mitigation
- Develop access and affordability strategies with milestones aligned to development stages
- For Rare Disease Programs, ensure actuarial risk and mitigation strategies are included in CAP processes
- Dedicate CIRM resources to partnerships with CA-DHCS, NIH, FDA, etc., to scale impactful programs
- Engage Alpha Clinics in oncology trials to accelerate access and streamline coverage analysis

Factors Impacting Access & Reimbursement Policy

PRICE

**HEALTH
GAIN**

**DEMAND
(PATIENT
POPULATION)**

VALUE ASSESSMENT

1. Are there specific options CIRM should prioritize or triage (e.g., near-term vs. long-term priorities)?
2. Are there opportunities missing from table that should be considered in the final SAF?

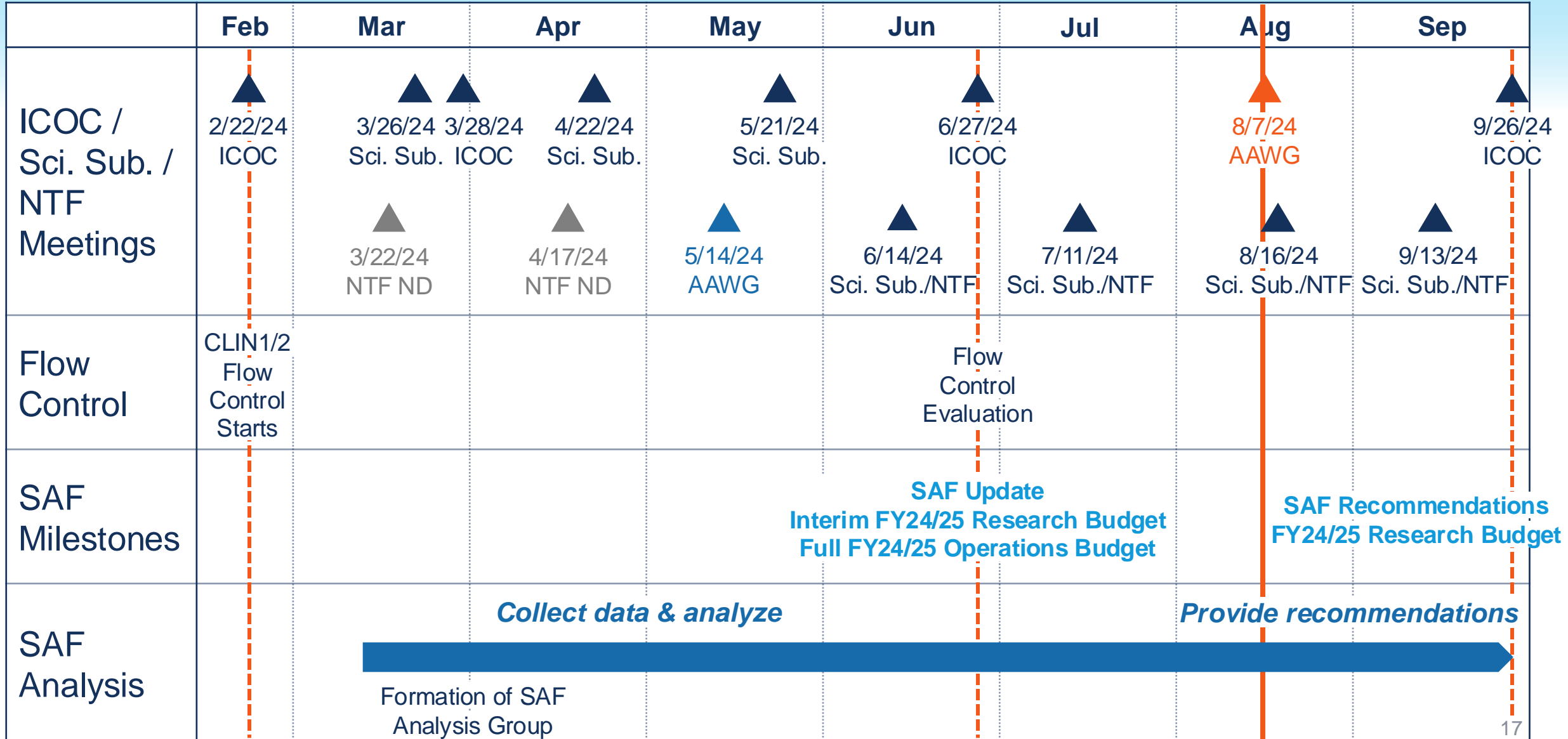
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TODAY



Meeting	SAF Topics
June NTF/Science Subcommittee	<ul style="list-style-type: none"> • 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	<ul style="list-style-type: none"> • 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	<ul style="list-style-type: none"> • 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 NTF/Science Subcommittee	<ul style="list-style-type: none"> • 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
August AAWG	<ul style="list-style-type: none"> • Present updates on Goal 5 and discuss associated data • Discuss potential recommendations for Goal 5
September NTF/Science Subcommittee	<ul style="list-style-type: none"> • Full SAF presentation: <ul style="list-style-type: none"> • Present updates based on feedback received on Goals 1, 2, 3, 4, & 5 • Present Goal 6 • Discuss overall recommendations in preparation for September ICOC
September ICOC	<ul style="list-style-type: none"> • Overall Presentation of SAF recommendations