

August 2024 AAWG



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Our Mission

Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world.





Agenda

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 Access and Affordability Strategy
4. Discussion

Patient Support Program

- 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

Community Care Centers of Excellence

- 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

NIH-RFI: Promoting Equity Through Access Planning

- New policy within its Intermural Research Program intended to promote access
- Includes drugs, biologics, vaccines or devices
- Include sublicenses and cost-plus purchasing agreements
- CIRM team and AAWG members drafted response

Strategic Allocation Framework

1. Background
2. SAF Overview
3. Goal 5 Access Strategy
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2022 – 2027

5 Year Strategic Plan



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 Center of Excellence



Provide Opportunity for All

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

Strategic Allocation Framework (SAF)

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, 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

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

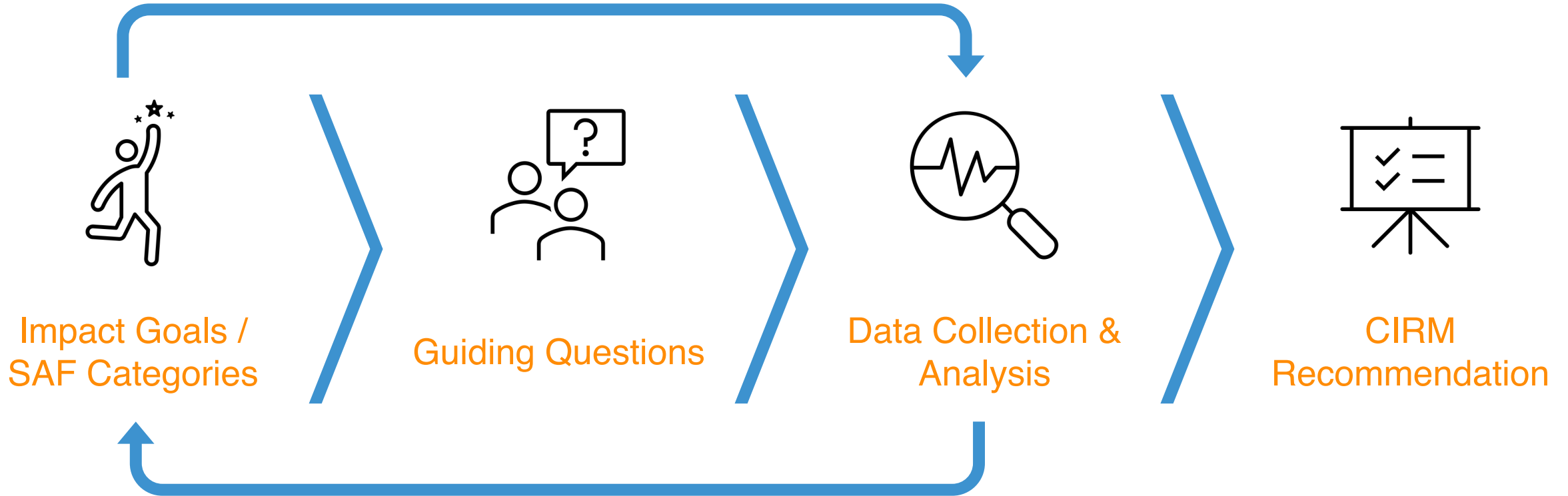
SAF I Design Questions

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 neurological disease budget of \$1.14B?

SAF I Process*

CIRM Staff



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

SAF I Categories

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

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 distinct diseases in California to late-stage trials, including a neurological condition, to significantly reduce morbidity and mortality

Accessibility & Affordability of CIRM-Funded Cell & Gene Therapies

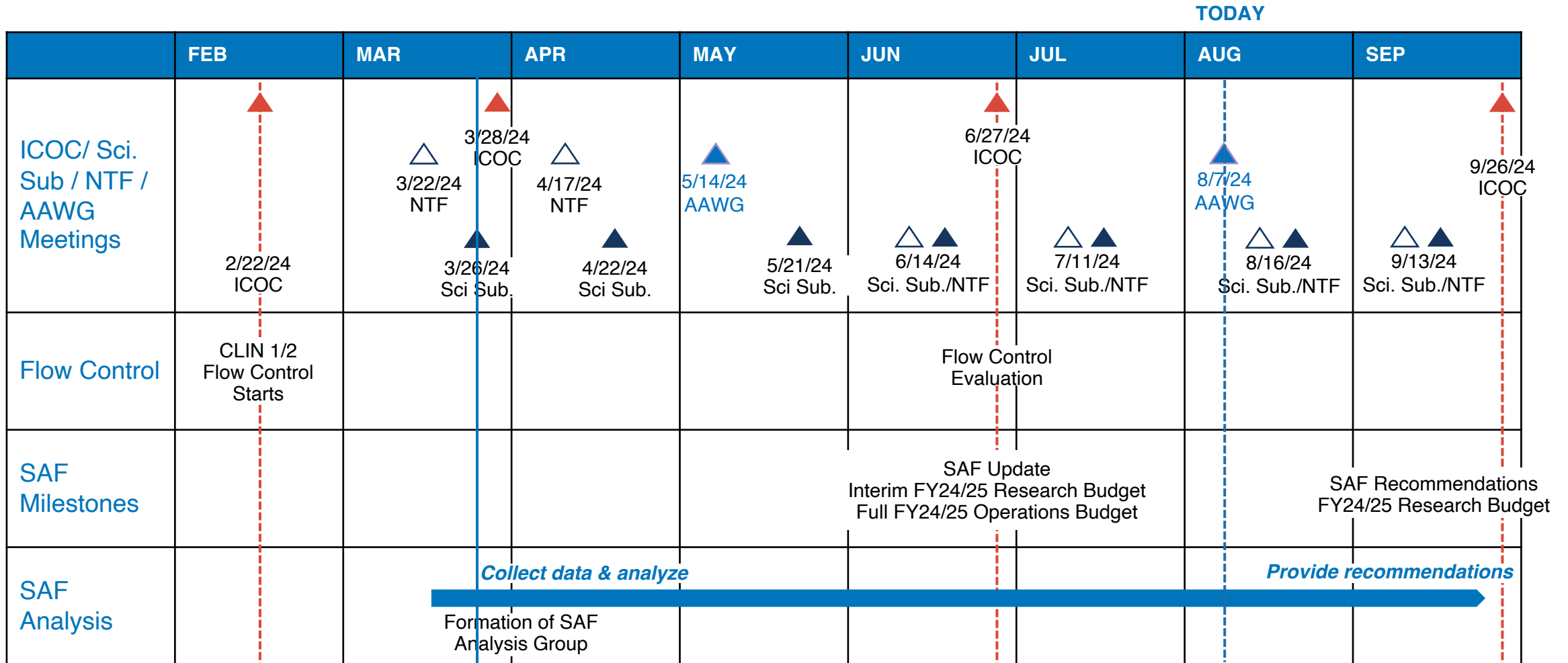
5. **Ensure** that **every** CIRM funded project completing a late-stage clinical trial has a strategy that enables access and affordability by all California patients, particularly underserved populations

Diverse Workforce Development

6. **Enhance** the integration and real-world application of training programs through strategic partnerships

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

Timeline



Goal 5

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

- Discussion

C I R M

CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE

Thank You



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