

SAF Update & Next Steps

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AAWG
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1. Provide background on Prioritization to AAWG
 - Introduce Goal 2
2. AAWG/NTF/Science Subcommittee coordination

1. Background

- Strategic Allocation Framework
- Process

2. Goal 2 – AAWG input

3. Timeline



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

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

CIRM needs to strategically allocate remaining resources to maximize its impact by considering available funds and reviewing past allocations

- 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
- Science Subcommittee (Sept '23) - Prioritization Kickoff Discussion (BM Fischer-Colbrie)
 - **Next Steps:** Ask for CIRM staff to develop an approach and recommendations for prioritization

Over the past few months, CIRM has developed a framework to provide strategic recommendations for funding allocation

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Structure

- Background & Rationale
 - Remaining Funds
 - Regenerative Medicine Landscape
- CIRM's Impact to Date
- Strategic Allocation Framework
- Proposed Timeline



Output

Recommendations for strategic priorities

Total Research Budget

From Prop 71 & Prop 14

\$7.64B



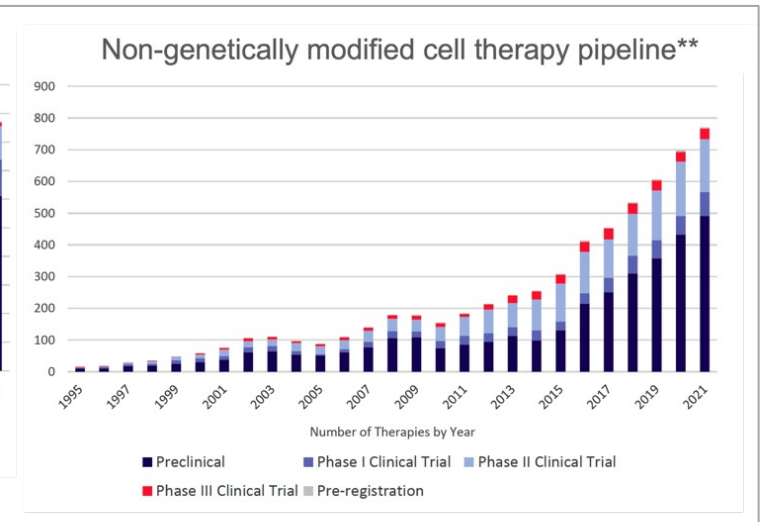
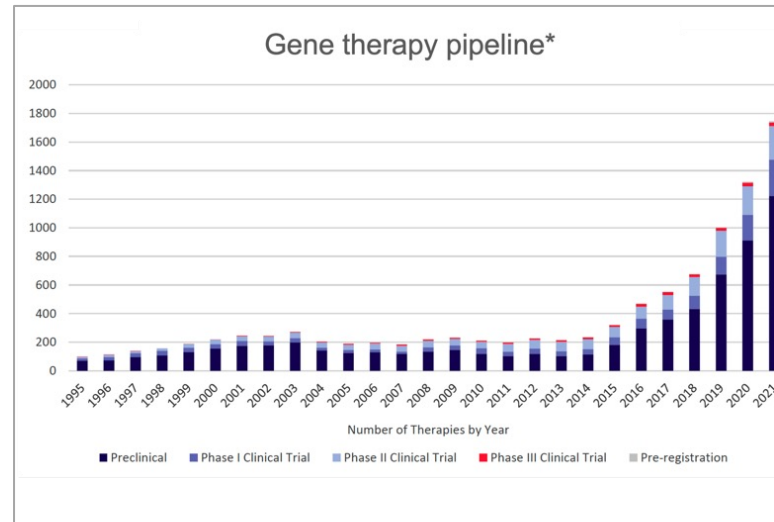
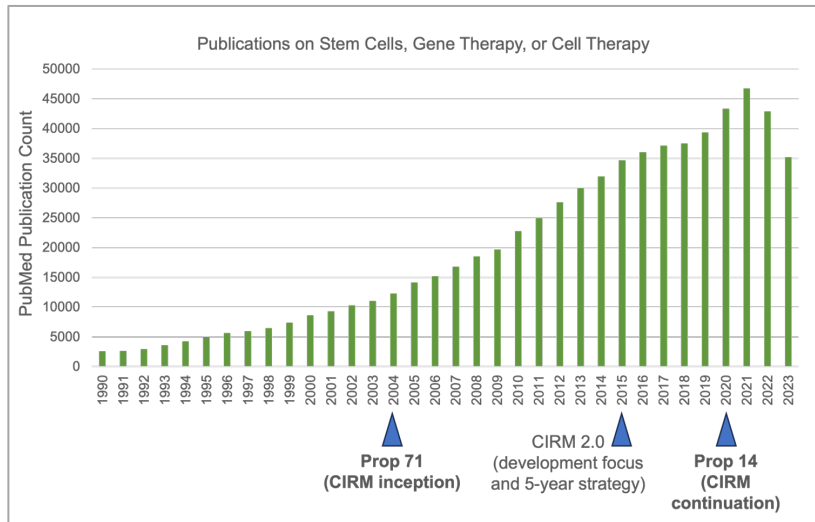
Current Research Allocation

Excluding expended/scheduled payments & approved allocations

Remaining Balance: **\$3.54B**

- For Neuro Research: **\$1.11B**
- Access & Affordability: **\$93.56M**

Exponential growth in the number of stem cell and genetic therapies companies, products, academic faculty, research projects and clinical trials from 2005 to 2022



The regenerative medicine field is expanding rapidly, increasing the need for strategic allocation in funding



Development of Cell and Gene Therapies

CIRM is committed to advancing cell and gene therapies from research to FDA marketing approval, while supporting patient access and scaling of manufacturing processes



Collaborative Networks for Discovery Research

CIRM is fostering collaborative ecosystems that leverage CIRM's investments, enhancing understanding of disease mechanisms and leading to discoveries that will push boundaries in treatment development



Training and Workforce Development

CIRM's educational initiatives integrate education with practical applications, creating a comprehensive network that connects training with real-world application and leverages CIRM-funded infrastructure



Advancements in Regenerative Medicine Technologies

CIRM supports research to improve regenerative medicine technologies ensuring the continuous evolution of the field



**Recommendations
will determine the
impact of CIRM
moving forward**

Determine:

- How can CIRM make the greatest impact on its mission?
- How might CIRM effectively allocate its remaining budget of \$3.54B?
 - How might CIRM effectively allocate its remaining **Neuro budget of \$1.11B?**

CIRM Staff



**Impact Goals /
SAF Categories**



**Guiding
Questions**



**Data Collection &
Analysis**



**CIRM
Recommendation**

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



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



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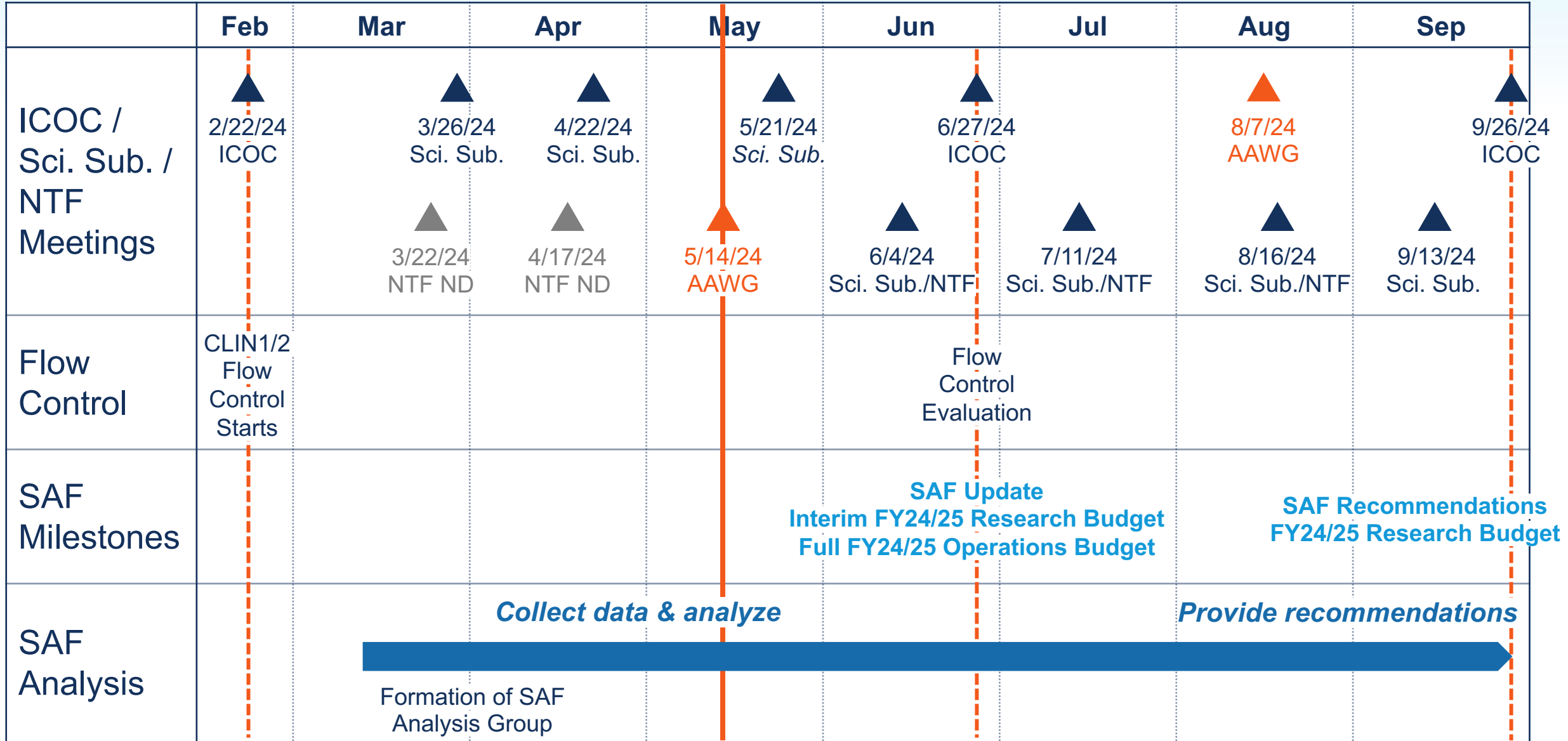


Goal #2: Ensure that **every** CIRM funded project completing advanced clinical trial has a strategy that enables access and affordability by all California patients, particularly underserved populations

1. What is the landscape of A/A for CGTs?
2. How can data from non-CIRM-funded programs be leveraged?
3. How should A/A strategies be implemented?

Timeline (slide 1/2)

TODAY



Timeline (slide 2/2)

	Sep	Oct	Nov	Dec
ICOC / Sci. Sub. / NTF Meetings	<p>▲ 9/26/24 ICOC</p> <p>▲ 9/13/24 Sci. Sub.</p>		<p>▲ TBD AAWG</p> <p>▲ TBD Sci. Sub./NTF</p>	<p>▲ 12/12/24 ICOC</p> <p>▲ TBD Sci. Sub./NTF</p>
Flow Control				
SAF Milestones	SAF Recommendations FY24/25 Research Budget			
SAF Follow Up	<p><i>Develop & Amend Concepts</i></p>			

Upcoming ICOC & Subcommittee Meetings

Meeting	SAF Topics
June NTF/Science Subcommittee	<ul style="list-style-type: none"> • Discuss summaries of NTF recent ND informative sessions (PD, ALS, AD, HD) • Review relevant questions and recommendations arising from the NTF summaries – 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 Goal 3 • Review relevant data associated with Goal 3 • Discuss potential recommendations for Goal 3
August NTF/Science Subcommittee	<ul style="list-style-type: none"> • Present updates based on feedback received on Goal 3 • Introduce Goal 1 and discuss associated data • Discuss potential recommendations for Goal 1
August AAWG	<ul style="list-style-type: none"> • Present updates on Goal 2 and discuss associated data • Discuss potential recommendations for Goal 2
September Science Subcommittee	<ul style="list-style-type: none"> • Full SAF presentation: <ul style="list-style-type: none"> • Present updates based on feedback received on Goal 1 and 3 • Present Goal 2 (from AAWG feedback) and Goal 4 together, discussing strategies and data relevant to both
September ICOC	<ul style="list-style-type: none"> • Overall Presentation of SAF recommendations



BACKUPS

Clinical (CLIN) Funding Opportunities

Program	Aim/Scope	Eligibility	Institution	Funding ¹	Co-Fund ²	Duration
CLIN1	IND Enabling	Pre-IND	Non-Profit	\$7.5M	0%	2 years ³
			For-Profit	\$5M	20%	
CLIN2	Clinical Trial	Active IND Phase 1	Non-Profit	\$12M	0%	4 years
			For-Profit	\$8M	30%	
		Active IND Beyond Phase 1	Non-Profit & For-Profit	\$15M	40%	
CLIN4	BLA Enabling	Active CLIN2/End of Ph2 Meeting	Non-Profit & For-Profit	\$12M	40%	4 years

1. Total CIRM-funded project costs; 2. Co-funding based on Allowable Project Costs (sum of CIRM funds requested plus the co-funding contribution by the applicant); 3. Up to 6 months additional for start up activities