

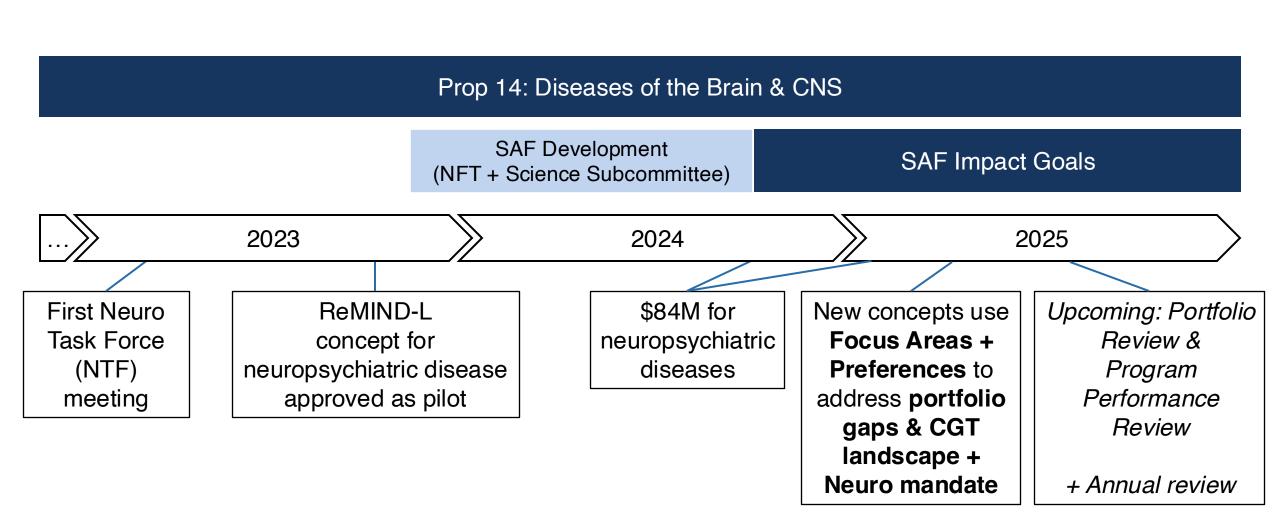
SAF ImplementationPhase 1: DISC4, DISC5, PDEV, CLIN2 Concepts

March 27, 2025





Preference Setting Builds on NTF and SAF recommendations





Agenda

- 1 Background and Concept Development Process
- 2 DISC5: New Concept
- 3 DISC4: Concept Updates
- 4 PDEV: New Concept
- CLIN2: Concept Updates



Background and Process

SAF Implementation Phase 1: DISC4, DISC5, PDEV, CLIN2 Concepts

- 1. CIRM's Impact Goals
- 2. Concept Development
 - a) Timeline
 - b) Process
 - c) Program Overview
- 3. Launch Timeline

SAF | CIRM's Impact Goals

Accelerating Discovery & Translation

- Catalyze the identification and validation of at least 4 novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California
- **Accelerate** development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies

Cell & Gene Therapy Approvals

- **Advance** 4-7 rare disease projects to BLA 3.
- **Propel** 15-20 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

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

SAF | CIRM's Impact Goals

Accelerating Discovery & Translation

- Catalyze the identification and validation of at least 4 novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California
- **Accelerate** development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies

Cell & Gene Therapy Approvals

- Advance 4-7 rare disease projects to BLA
- **Propel** 15-20 therapies targeting diseases affecting Californians to late-stage trials 4.

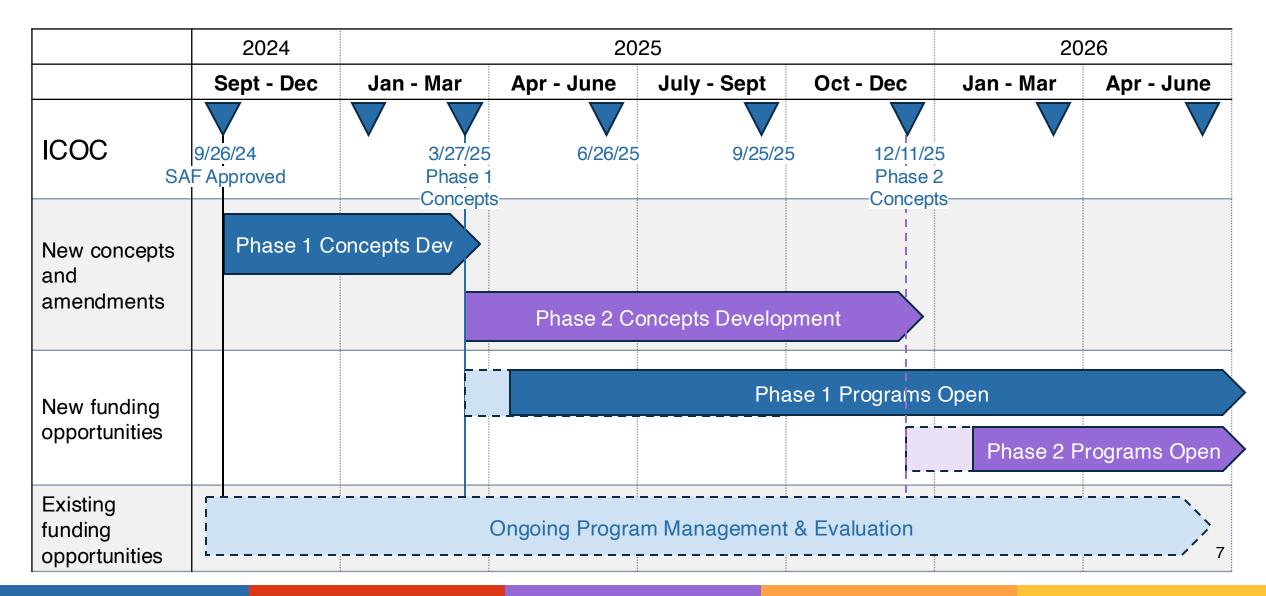
Accessibility & Affordability of CIRM-Funded Cell & Gene Therapies

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

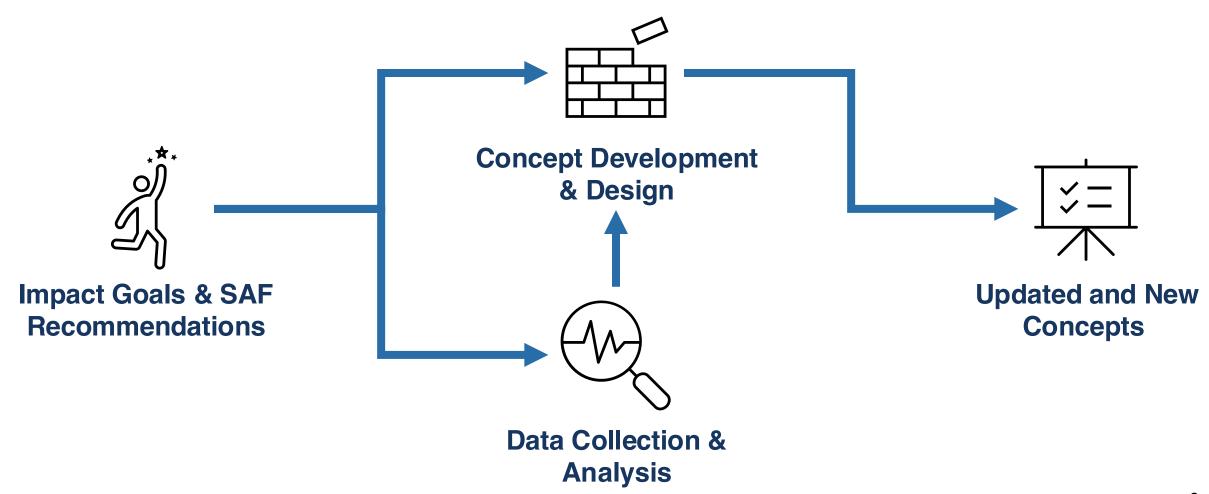
Diverse Workforce Development

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

SAF Implementation Timeline (in development)



Concept & Program Development Process



Overview of New & Updated Concepts

	DISC5	DISC4	PDEV	CLIN2
Focus	Exploratory / Innovative Basic Science	Large Multidisciplinary Collaborations	Preclinical Through IND	Clinical Trials
Preferences	N/A	Systems Biology Focus Areas	Candidate Preferences Aligned to SAF Goals	Candidate Preferences Aligned to SAF Goals
Award Amount	\$2.5M	\$13M	\$13M	\$8-15M
# Awards per Year	15-20	6	12-21	9-16

Concept Launch Timeline

