Funding Area Preferences

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Science Subcommittee
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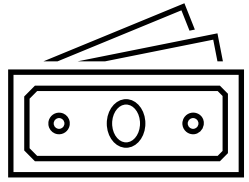
CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Presentation Overview

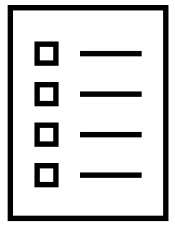
1. Context & Objectives
2. Guiding Principles
3. PDEV & CLIN2
4. DISC4
5. Closing

Proposition 14 & Where We Are Now



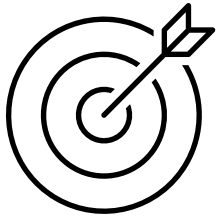
Finite Funds

- Finite remaining runway
- CIRM cannot fund everything



Expanded Mandates

- CNS
- Access & affordability



Goal

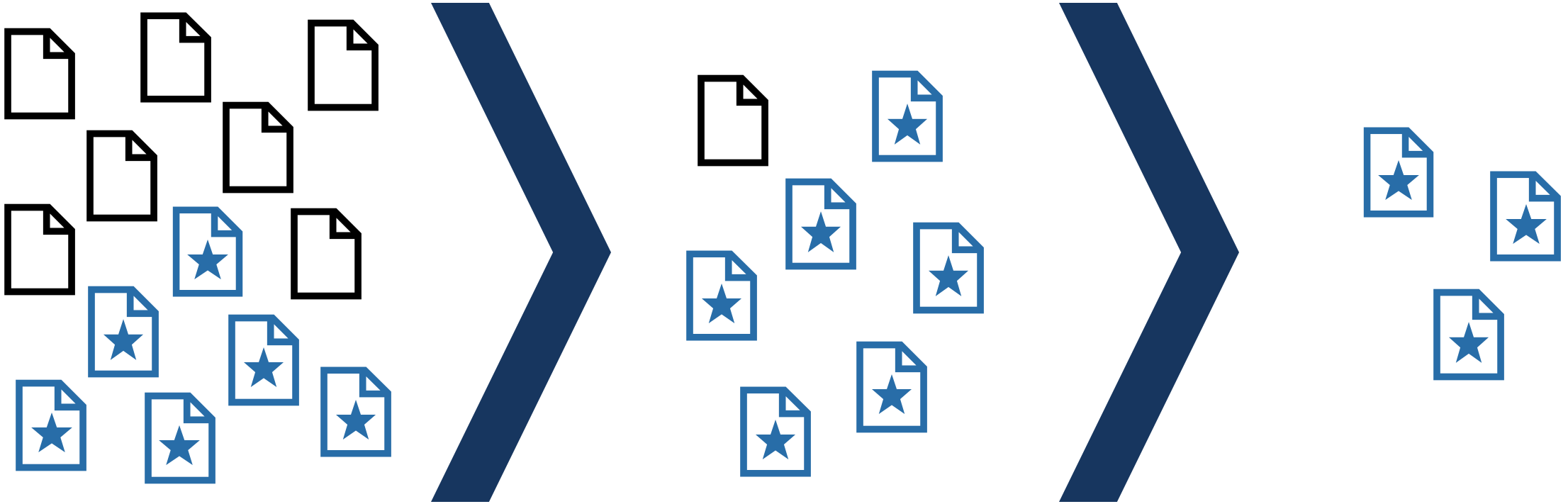
- Translate public investment into **therapies reaching patients** within CIRM's lifetime

Challenge: CIRM receives **more applications than it can fund**

Preferences Help Achieve Therapies in CIRM's Lifetime

Preferences

Review



Too many applications

Applications enriched for impact

Impactful applications with the highest scientific merit

What We Have Heard

- Anxiety that proposals were triaged without scientific review
- CIRM is not funding progression events
- Perception of modality bias
- Desire for clarity on qualification scoring, preferences, and intent
- Misunderstanding of what preferences do / don't do
- Need for clearer communication & broader reach

It is critical for CIRM to **clarify how actions taken** so far will lead to **therapies for patients** and achieving its mission

Today's Objectives

- Review **rationale for preferences**
- **Share learnings** from first funding cycles
- **Identify portfolio analyses needed** to refine preferences

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Guiding Principles for Preference Setting

Guiding Principles

1. Offer potential for transformative clinical impact
2. Address bottlenecks to access, affordability, & translational feasibility
3. Fill critical funding gaps & advance CIRM's statutory mandates
4. Can realistically achieve key regulatory and development path within CIRM’s finite runway
5. Address diseases affecting Californians
6. Diversify CIRM’s active award portfolio

PDEV Pre-Submission Rubric

Preference	Points
At least one of the following: • PSC-derived therapies • In vivo gene therapies • Diseases of the CNS	3
• Non-viral nucleic acid delivery	1
• Pre-IND or INTERACT meeting conducted	1
• Progression from DISC2 or TRAN1	1
• Targeting disease area under-represented in CIRM active awards portfolio	1
• Novelty of therapeutic approach compared to CIRM active awards portfolio	0-2

PSC: Pluripotent Stem Cell; CNS: Central Nervous System; IND: Investigational New Drug Application; INTERACT: INitial Targeted Engagement for Regulatory Advice on CBER/CDER ProducTs

CLIN2 Qualification Rubric

Preference	Points
• PSC-derived therapies	1
• Diseases of the CNS	1
• In vivo genetic therapy	1
• Non-viral genetic therapy	1
• Accelerated regulatory designation (RMAT, Breakthrough, Fast Track)	1
• Progression from earlier stage CIRM award	1
• California Organization	1
• Pivotal trial	2

PSC: Pluripotent Stem Cell; CNS: Central Nervous System; RMAT: Regenerative Medicine Advanced Therapy

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PDEV Cycle 1 Results

Total Pre-Submissions:
168

Invited to Full Application & Review:
33

Top 20% invited
- 97% of invited met 3-4 preferences
- 42% of uninvited met 0-1 preferences

Funded:
12

- Increase in proposals with innovation & scalability
- Over half are CIRM portfolio progressions
- Enriched for less represented disease areas

Awarded programs **reflect preferences** and CIRM's preclinical **portfolio is now better balanced** across modality, disease, & Prop 14 CNS priorities

PDEV Cycle 2 Results

Total Pre-Submissions:
126

Invited to Full Application & Review:
23

Top 18% invited:
- 100% of invited met 3-5 preferences
- 39% of invited would be CIRM portfolio progressions if funded
- 33% of uninvited met 0-1 preferences

Recommended for Funding:
TBD

- GWG Review scheduled for March 2026

23 invited applications represent broader disease areas and include more cancer & immunology

CLIN2 Cycle 1 Results

Total Applications:
23

- Ranked by preference points

Advanced to Full Review:
7

- All 7 had 3-4 preference points
  - All targeted CNS
  - 6 were pluripotent stem cell or in vivo genetic therapy
  - 4 had advanced designations

Funded:
4

- All use next-generation or platform modalities
- All have feasible delivery paths consistent with A&A strategy
- 3 are CIRM portfolio progressions

The system worked directionally as intended, but **scoring weights need refinement**

CLIN2 Cycle 2 Results

Total Applications:
21

- Ranked by preference points

Advanced to Full Review:
7

- All 7 advancing had 3-4 preference points
  - 4 target CNS
  - 5 are CIRM portfolio progressions
  - 5 are in vivo genetic therapies or PSC-derived therapies

Funded:
TBD

- GWG Review scheduled for January 27, 2026

Cycle 2 results are pending GWG Review & ICOC/ARS approval

PDEV & CLIN2 Cycle 1 | Summary

- Awards in both PDEV and CLIN2 reflect the innovation, readiness, Access & Affordability, and CNS criteria in the Program Announcements
- Applications advancing were largely CIRM progressions that also met multiple preferences
- The resulting portfolio now includes more candidates with feasible, scalable modalities that strengthen Access & Affordability potential

Caveats

- These are preliminary signals
- Portfolio-level impact cannot be assessed yet

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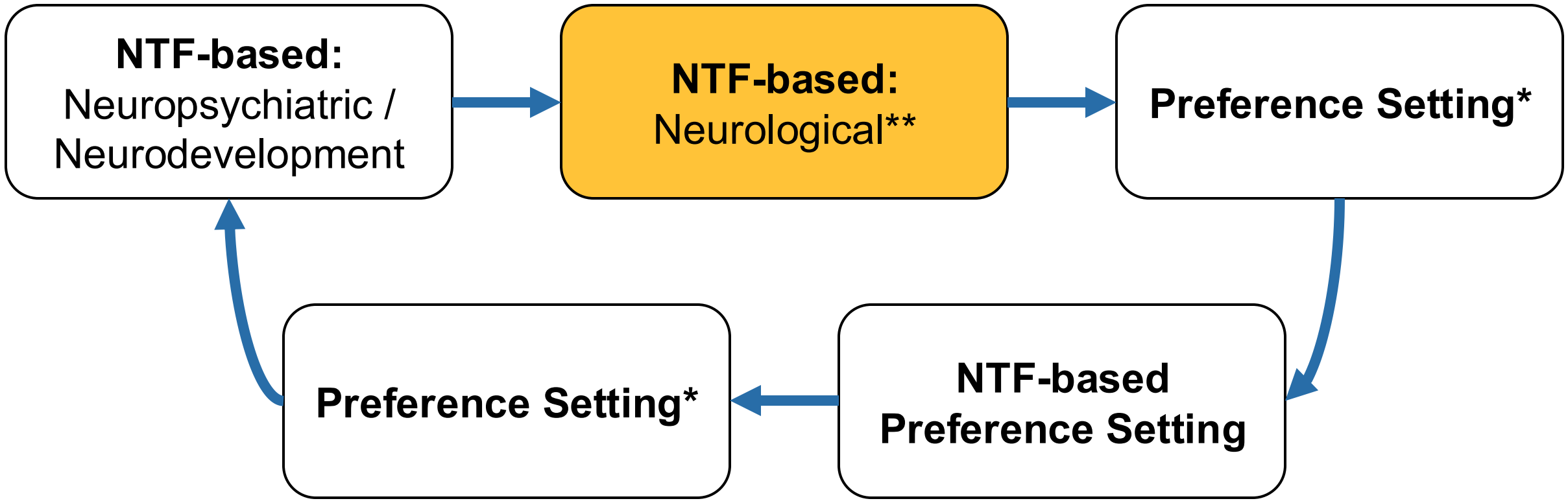
What We Set Last Year

- CIRM staff proposed alternating preference cycles for DISC4, with Neuro every other year
- The Board approved Neurological Disease as the preference for the FY25/26 DISC4 cycle; the program was open to all comers

DISC4 Preferences | Alternating Neuro Cycles

Enabling NTF Prioritization while allowing other diseases to use this structure



*All cycles will be open to all-comers with alternating preferences determined by either NTF or by the Board each year based on portfolio analyses
** FY25/26, ICOC selected broad Neuro preference– In first round, Neuro preference had a weight of 36% overall

DISC4 FY25/26 Preference Scoring

Criteria	Description (listed in program announcement)	Weight (%)
Preference Topic: Neuro	Does the project align with the cycle-specific preference topic?	36%
Relevance to human disease biology	Does the project hold strong relevance for understanding or addressing human diseases?	24%
Cross disciplinary and systems biology	Does the project integrate cross-disciplinary approaches?	24%
Stem cell or genetic research innovations	Does the project incorporate innovative approaches particularly in stem-cell or genetic research?	16%

DISC4 Cycle 1 | Pre-submission Results

Total Pre-Submissions:
138

- **86%** met the **Neuro** preference

Invited to Full Application & Review:
24

- **100%** invited **met the Neuro preference**, covering range of diseases
- Large range of mechanisms & approaches represented

Recommended for Funding:
TBD

- GWG Review scheduled for **February 2026**

Preferences enriched the pool: all invited applications aligned with the Neuro preference while maintaining scientific diversity

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What We Learned

- **Initial signals from preference setting**
  - Applications that advanced to GWG review met multiple preferences
  - High percentage of CNS projects funded for CLIN2
  - Wide range of disease areas and modalities funded for PDEV
  - High percentages of funded projects are CIRM progressions (>50% PDEV, 75% CLIN2)

January presentation **tees up the questions** for **Board**; **March** brings the **portfolio** analysis and any potential preference refinements based on Board guidance

Planned Analyses for March ICOC

- Definition & examples of what we categorize as innovation, progressions, and disease areas
- Active portfolio & application cycles broken down by disease area, modality, progression status, and how CIRM is driving innovation

Does the Science Subcommittee request **any other portfolio analyses** to **inform preference setting**?